Paying for value: international developments

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UK developments in drug pricing

• “We recommend that Government reform the PPRS replacing current profit and price controls with a value based approach to pricing to ensure the price of drugs reflect their clinical and therapeutic value to patients and the broader NHS.”

OFT, February 2007
Capturing value: payers’ tools

- **Direct**
  - Value-based pricing
- **Indirect**
  - Comparative effectiveness information
  - Coverage with evidence development / “only in research”
  - Risk Sharing Schemes
“In the case of promising interventions not yet supported by sufficiently robust evidence to justify an unqualified recommendation, the Institute will:

• recommend that further research is carried out to see whether the potential promise of the intervention can be realised,

• indicate in broad terms the questions this research should address and

• advise clinicians that, in the meantime, they should only use the new intervention as part of a well-designed programme of research intended to answer these questions”

OIR examples: public money

“Laparoscopic surgery should only be undertaken for colorectal cancer as part of a randomised controlled clinical trial” 2000

“Laparoscopic resection is recommended as an alternative to open resection for individuals with colorectal cancer…” 2006

“PDT is recommended for people who have wet ARMD with mostly classic subfoveal CNV only as part of a clinical study designed to provide useful information on the effectiveness of the treatment.” 2003

NHS R&D-funded Verteporfin PDT cohort study*

Review pending; final results anticipated 2009

*Mhttp://www.lshtm.ac.uk/hsru/vpdt/
OIR examples: private money

“The use of taxanes for adjuvant treatment of early breast cancer should be limited to randomised clinical trials” 2000, 2001*

“Docetaxel […] is recommended as an option for the adjuvant treatment of women with early node-positive breast cancer” 2006

“Paclitaxel […] is not recommended for the adjuvant treatment of women with early node-positive breast cancer” 2006

No new evidence of clinical or cost effectiveness compared to standard NHS practice submitted

*Taxanes were not licensed for this indication at the time but had both received license for use as adjuvant Rx by 2006
NICE Risk Sharing Schemes: MS

• “On the balance of their clinical and cost effectiveness neither b-IFN nor glatiramer is recommended for the treatment of MS in the NHS…Unless further evidence emerges … the cost-effectiveness of these medicines can only be improved if there is a significant reduction in the total cost of their acquisition by the NHS.”

*NICE 2000*

• National risk sharing scheme (approx. 9,000 patients) established by DH in collaboration with manufacturers “equivalent in effect to the directions relating to positive recommendations from NICE”

*Health Service Circular 2002/004*

• Review planned Nov 2004; deferred to Nov 2006; current status: pending…

HIF OCT 07
NICE Risk Sharing Schemes: bortezomib

The DRAFT decision, subject to appeal:
• Treat patients at first relapse for up to 4 cycles
• Measure serum M protein (response marker)
• Continue treatment when >50% reduction in M protein (complete or partial response).
• Seek full rebate (cash or stock) for non-respondents by J-C
• £20,700/QALY
NICE Risk Sharing Schemes: bortezomib

- Two rounds of public consultation
- Multiple company submissions
- Independent re-analyses of submitted data by academic group and in-house NICE technical team
- Appeal upholds key manufacturer grounds
- More analyses on risk sharing scheme and draft guidance re-issued

(Some of the) scenarios modelled using incremental analysis
- Adjusting for cross-over (APEX trial)
- Stopping rule: 3 or 4 (or 5) cycles
- With and without rebate from J-C
- Vial sharing practices
- £/QALY or £/LYG
- Treatment adverse event costs
- Response criteria: EBMT or serum M protein (or urine BJ)
- First or subsequent relapse
- Responder groups: complete, partial, minimal
- Monotherapy or combination therapy (HDD) - *not* modelled (outside license)
NICE Risk Sharing Schemes: bortezomib

It’s not over yet! Collecting and using the data

- Simple and low administrative burden; single page fax back form
- Directly run between manufacturer and provider hospitals
- Cost of requesting rebate and IPD collection borne by NHS; not accounted for in CEA
- No academic, government, NICE or professional input
- Manufacturer access to both aggregate and (anonymised) IPD data for non-responders
- Government reserves right to access the data
- 2010 NICE update: subgroup analysis; optimal stopping rule; real world comparator analysis; scheme evaluation
Uncertainty is a political problem

- CEA closer to licensing: NICE single technology appraisal
- Methodological fix
  - Value of Information analysis
  - NICE Methods Manual - Dec 2007
- Ongoing debate
  - Evidence of lack of effectiveness vs. lack of evidence of effectiveness
  - Who’s responsible for producing the information?
  - Should/can we say ‘no’ when we do not know?
What does the public think? Citizens’ Council

• In what circumstances is it justified for NICE to recommend that an intervention is used only in the context of research?
  – Feasibility
  – Access
  – Timeliness
  – Value for money of research
  – Implications of positive decision

• “Patients would be reassured to know that clinicians and the healthcare system in general could face up to uncertainty, and were confident enough to deal with it in a mature, scientific way, and avoid wasting money on unproven technologies”
Health research reform in the UK

- “….funding should be identified and formal arrangements established between the NHS R&D Programmes and NICE in order to implement NICE recommendations calling on the NHS to use health interventions in a research context.”
  Sir David Cooksey, Dec 2006
- New “critical path” for drug development - NICE and MHRA collaboration
- Fully endorsed by Gordon Brown in 2006 pre-Budget report and CRS
- New coordinating unit: OSCHR: £1.1b p.a.
Health research and the UK IT experiment

- UKCRC clinical trial network: over 70 pharma trials currently running on disease specific NHS networks
- Connecting for Health: 4 simulations
  - **Surveillance**: disease and health surveillance, drug and devices safety, decision support systems, medical errors reporting
  - **Clinical Trials**: population profiling, power calculations, recruitment assistance, data collection and analysis, post-marketing surveillance
  - **Prospective Tracking of a Known Cohort**: UK Biobank: blood-based resource; 500,000 people 40-70 yrs old; blood markers, sociodemographic data, history, SEG; prospective follow-up and link with NHS records
  - **Observational Epidemiology**: retrospective cohorts and case control studies based on routine data; record linkage requirements
Going wider: providers and physicians

Payment by Results: the “tariff” for providers
• Based on survey of providers: historical costing data
• Adjusted for case mix and need
• Tariff “uplift” for NICE guidance
• Ongoing debate on whether to move to normative tariff

Quality and Outcomes Framework: primary care physicians
• Little evidence of outcomes or cost effectiveness
• Inaccurate baseline
• Targeting underuse - no focus on overuse or misuse
• Indicators under review
Expanding NICE-type evaluations in...

- “areas of treatment where significant resources are involved
- new policy areas that impact on clinical practice to test whether they produce health gains.”
  King’s Fund, Spending on healthcare: how much is enough? Feb 2006

- service delivery, in chronic care
- centrally-set targets: waiting lists
- provider reimbursement: PbR

- “[…] a priority for future spending plans should be to expand the remit of NICE to review all new NHS drugs and treatments by efficacy value within a reasonable timeframe.”
  ippr, Great expectations: achieving a sustainable health system, Sep 2007
Professional-led initiatives

- Antihypertensives: head-to-head trial (ANBP2) - with PBAC funding support (Australia)
- Extra Corporeal Membrane Oxygenation trial - Society of Neonatologists (UK)
- Hip registry - Association of Orthopaedic Surgeons (UK) with NICE and NHS support
- Avastin vs. Lucentis trial - Ophthalmic Surgeons (UK) with NHS R&D and NHS Commissioners’ support
International examples

• France
  – EUnetHTA: EU Commission initiative to promote evidence generation for new drugs
  – Drug pricing based on HAS determined tiers: currently only clinical effectiveness but economic evaluation being considered

• Germany
  – New Social Insurance Code: ceiling price on innovations
  – Economic evaluation required by law (2007) for IQWiG

• Korea
  – Pricing informed by CEA introduced earlier this year (HIRA)

• Italy
  – 5% tax on marketing to fund comparative effectiveness research
  – Used funding to undertake head-to-head trial of b-IFN against azathioprine; the latter is better value
UK government response

“We agree with the OFT that it is time to develop a pricing system which is fit for purpose for the twenty first century. We must ensure that any future pricing scheme delivers value, rewards innovation and ensures a fair deal.”

Competitiveness Minister Stephen Timms,
London, August 2007
In the press

**FT.com World**

**REUTERS**

**ANALYSIS-UK turns screw on drug prices in hunt for value**

**THE INDEPENDENT**

**Drug firms accused of 'ripping off' the NHS**

By Colin Brown, Deputy Political Editor
Published: 21 February 2007
Thank you
Additional slides
Better value can be more expensive

Webb and Walker, Lancet 2007
Statutory right

• “When appraising health technologies, NICE would be able to recommend:
  1. the routine use of an intervention in the NHS, for all or specific licensed indications or patient subgroups
  2. against the use of the intervention in the NHS because of inadequate evidence of effectiveness, or, cost-effectiveness,
  3. the use of the intervention in the NHS only in the context of appropriate research”

Pending NICE “only in research”: 1999-2006 (cont.)

- Examples of “only in research” recommendations where review is pending awaiting new information:
  - Temozolomide for brain cancer (2001):
  - Review in 2007 to account for findings of BR12 MRC trial
  - Imatinib for chronic myeloid leukaemia (2002):
  - Review in 2009 pending relevant trials reporting in Dec ‘07 and ’08
WHO review of GLs 2007

• “Use only in the context of research” should be an explicit option for inclusion in recommendations and there should be clear guidelines on when it is appropriate to consider and recommend the inclusion of this sentence.
“Only in research” for interventional procedures

- Almost half (46%) of procedures reviewed by IP, recommended with “special arrangements for clinical governance, research and audit”
  - Large case series and registers are needed to provide evidence of long term efficacy and safety

Implications of getting it wrong and...

...and saying “yes” instead

- Access to unproven and potentially harmful or ineffective interventions granted
- Relevant research is hindered
- NHS encourages “bad” value innovation
- NICE wastes limited resources
- Credibility is compromised when a “yes” decision is reversed

...saying “no” instead

- Patients are denied access to promising and potentially effective technologies
- Delays in building the evidence base
- Innovation that adds value is hampered and commercial R&D investment discouraged
International examples - ii

- Australia
  - ANBP2 trial; one-off PBAC involvement
- Japan
  - Innovation premium; non-transparent price setting by government but ongoing initiatives to consider cost-effectiveness