

Restricting Access to Healthcare: Is NICE Too Nasty?

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Outline of Presentation

- Some background on NICE
- NICE's achievements to date
- Comparison of NICE with other similar entities
- Analysis of recent developments at NICE
- Conclusions

Disclosure of Interests

- The Centre for Health Economics receives funding from the National Institute for Health and Clinical Excellence (NICE).
- I undertake consulting in the field of health technology assessment, both for governments and companies.
- I am Chair of one of NICE's Guideline Review Panels.

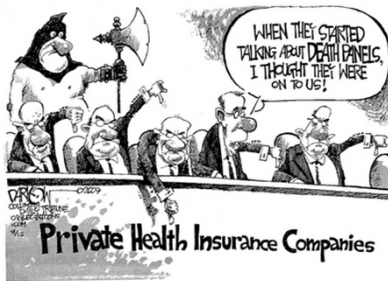
Disclosure of Interests (cont.)

Or, in the words of *Fox News*....

.....I serve on one of NICE's

DEATH PANELS!!!!

US Death Panel



National Institute for Health and Clinical Excellence (NICE)

- Created in 1999
- A Special Authority within the National Health Service (NHS)
- Remit is to consider 'clinical and cost-effectiveness'
- Programmes of work in:
 - health technology appraisal
 - new interventional procedures*
 - clinical guidelines
 - public health interventions

* Efficacy and safety only

Technology Appraisals versus Clinical Guidelines

- **Technology appraisals**

Deal with new technologies; narrow focus; many concern a single technology (eg a drug in a given indication); mandatory on the NHS.

- **Clinical guidelines**

Deal with existing care; broader focus; aim is to improve existing care patterns; adoption is voluntary.

NICE's Procedures

- Remit received from the Department of Health
- Scoping exercise undertaken
- Submissions invited from key sponsors or manufacturers of the technology
- Independent review of the evidence
- Guidance developed (by an expert group)
- Guidance issued to the National Health Service (NHS): mandatory for technology appraisals
- Monitoring of guidance and review (3 years)

NICE's Output to Date

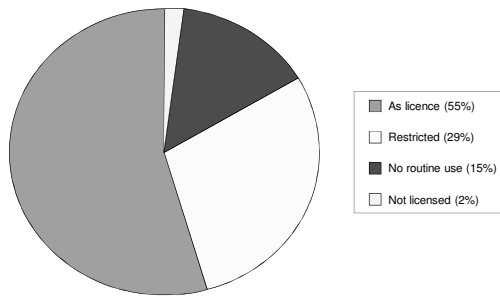
- Interventional procedures (320)
- Technology appraisals (184)
- Clinical guidelines (100)
- Public health appraisals (22)

NICE's Guidance on New Cancer Drugs: May 2000-March 2008: Methods

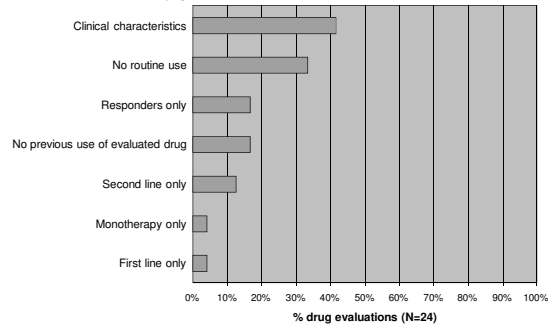
- Data sources
 - NICE published appraisals on cancer drugs
 - EMEA/MHRA licences/SPCs
- Data extraction
 - Drug, indication, recommendations
 - Stated justifications: uncertainty; methodological issues; trial evidence; ICER
- For each drug evaluation
 - compare recommendation with licence
 - classify recommendation as: licence; restricted; no routine use; not licensed
- Restricted/no routine use
 - 24/55 drug evaluations
 - reasons for restrictions explored

Mason A and Drummond M. *Eur.J.Cancer* 2009; 45: 1188-92.

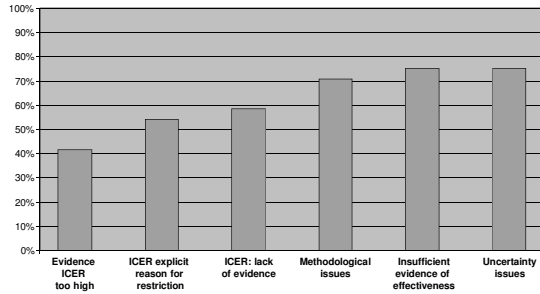
NICE Cancer Recommendations % cancer drug evaluations (N=55)



NICE Cancer Recommendations: Types of restriction



Reasons for NICE Restrictions: % drug evaluations (N=24)

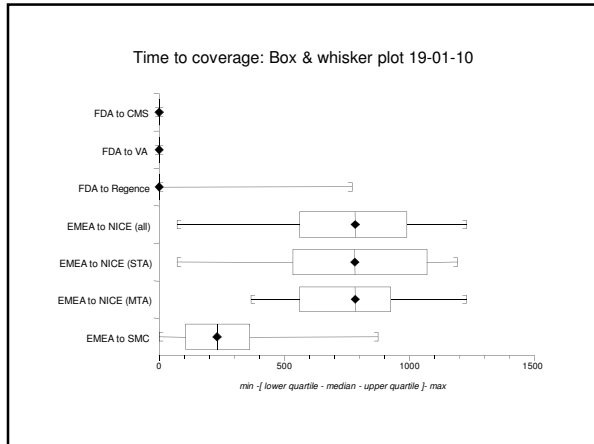


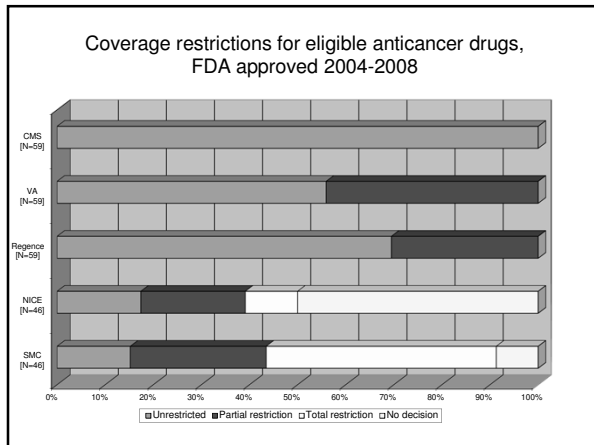
NICE's "Waiting List" (March, 2008)

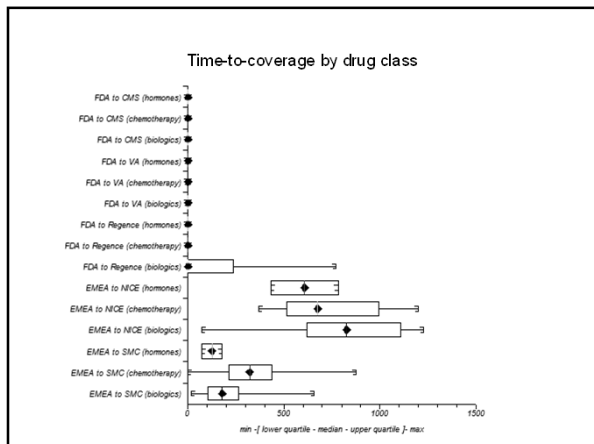
DRUG	TUMOUR TYPE	UK LICENCE DATE
bevacizumab	Breast cancer (advanced & metastatic)	12/01/2005
bevacizumab	Lung cancer (non-small cell)	21/08/2007
bevacizumab	Renal cell carcinoma	14/12/2007
capecitabine	Pancreatic cancer	?
cetuximab	Colorectal cancer	29/06/2004
cetuximab	Head and neck cancer	29/03/2006
cetuximab	Lung cancer (non-small cell)	?
dasatinib	Acute lymphoblastic leukaemia	20/11/2006
dasatinib	Chronic myeloid leukaemia	20/11/2006
erlotinib	Lung cancer (non-small cell)	19/09/2005
gefitinib	Lung cancer (non-small cell)	?
irinotecan	Colon cancer (adjuvant)	?
lapatinib	Breast cancer (advanced or metastatic)	positive opinion: 13/12/2007
lenalidomide	Multiple myeloma - lenalidomide	14/06/2007
liposomal muramyl tripeptide phosphatidyl ethanolamine	Osteosarcoma (newly diagnosed, non-metastatic, resectable)	?
nilotinib	Acute lymphoblastic leukaemia	19/11/2007
nilotinib	Chronic myeloid leukaemia	19/11/2007
sorafenib	Renal cell carcinoma	19/07/2006
sunitinib	Renal cell carcinoma	19/07/2006
temozolomide	Advanced and metastatic melanoma	?
temsirolimus	Renal cell carcinoma	19/11/2007
topotecan	Relapsed small cell lung cancer	13/01/2006

A Recent Controversy

- In August 2008, NICE published its Appraisal Consultative Document on four new drugs for treating advanced renal carcinoma: *bevacizumab*, *sorafenib*, *sunitinib*, *temsirolimus*.
- It recommended that none of the four drugs should be used in the NHS on the grounds that they were not cost-effective.
- Oncologists and patient organizations were outraged, since these drugs are widely used in many other countries and offer benefit to patients for whom no other effective treatments are available.







Options for the US

' We have a choice: do we use science to help us reach a consensus on what we are willing to pay for new therapies and innovation, or do we leave individual patients to wrestle with the skyrocketing costs of cancer care and treatment determined by their ability to pay'

Malin J, Geffen D. Editorial *JCO* 2010; 28. 9967

Comparing HTA Entities

- Range of technologies evaluated
- Types of evidence considered
- Quality of the review process
- Level of stakeholder involvement
- Transparency in HTA processes
- Communication and implementation of HTA results

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Key principles for the improved conduct of health technology assessments for resource allocation decisions

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Range of Technologies Evaluated

- In many jurisdictions, the HTA effort focuses on drugs, usually because of the remit the HTA entity has been given (eg the CvZ in the Netherlands).
- Consideration of a broader range of technologies is more likely to lead to overall efficiency and gives a 'level playing field' (eg NICE).

Types of Evidence Considered

- Most jurisdictions issue guidelines for submissions of evidence.
- For *clinical* evidence, some entities (eg IQWiG in Germany) place a major emphasis on RCTs.
- Others (eg NICE) recognize the relevance of observational data and economic modeling.
- Very few entities pay much attention to items such as productivity gains, or patient/family costs (TLV in Sweden is an exception).

Quality of the Review Process

- Some entities review manufacturers' submissions, and other evidence, in-house, others (eg NICE) commission independent expert reviews.
- Good quality review, using explicit criteria, is critical to the integrity of the HTA process.
- Transparency is important, as mistakes are made and need to be rectified!

Level of Stakeholder Involvement

- Varies from extensive (eg NICE) to very limited (eg PBAC in Australia).
- Most HTA agencies allow company submissions of evidence.
- Other examples of involvement include:
 - scoping of the assessments (eg. choice of the comparators, outcomes to be considered)
 - commenting on draft reports
 - appeals against recommendations

Transparency in HTA Processes

- Transparency is necessary for understanding the criteria used, the analyses conducted and the reasons for any recommendation.
- Mechanisms can be developed to protect commercial-in-confidence data.
- Transparency may be more of a challenge in the context of private payers.

Communication and Implementation of HTA Results

- Important to distinguish (as NICE does) between the *assessment* (ie science) and the *appraisal* (ie decision-maker's values).
- In single-payer, public healthcare systems HTA may lead to global recommendations.
- In systems with multiple payers, a general HTA 'finding', or a global recommendation, does not make sense.

Key Principles: How Does NICE Shape Up?

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POLICIES

Are Key Principles for improved health technology assessment supported and used by health technology assessment organizations?

The International Working Group for HTA Advancement

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Abstract: An group—the International Working Group for HTA Advancement—convened a global forum to discuss the current state of health technology assessment (HTA) in 2008. The group's goal was to identify key principles for HTA and to investigate the extent to which these principles are supported and used by health technology assessment organizations. The group identified 15 key principles for HTA and investigated the extent to which these principles are supported and used by health technology assessment organizations. The results of this investigation are presented in this paper.

Support and Use of Key HTA Principles by NICE

Key Principle:	NICE (UK) Inception: 1999
Structure of HTA program	
1. The goal and scope of the HTA should be explicit and relevant to its use	++
2. HTA should be an unbiased and transparent exercise	++
3. HTA should include all relevant technologies	++
4. A clear system for setting priorities for HTA should exist	++
Methods of HTA	
5. HTA should incorporate appropriate methods for assessing costs and benefits	++
6. HTAs should consider a wide range of evidence and outcomes	++
7. A full societal perspective should be considered when undertaking HTAs	++
8. HTAs should explicitly characterize uncertainty surrounding estimates	++
9. HTAs should consider & address issues of generalizability & transferability	++

Note. "+" signifies that the organization "supported" the principle in question in written guidelines or other form, regardless of whether they actually follow it. "++" means that the organization "implemented" the principle in published reports and decisions based on these reports demonstrate adoption of the specific principle. NICE = National Institute for Health and Clinical Excellence.

Support and Use of Key HTA Principles by NICE (contd)

Key Principle:	NICE (UK) Inception: 1999
Processes for conducting HTA	
10. Those conducting HTAs should actively engage all key stakeholder groups	++
11. Those undertaking HTAs should actively seek all available data	++
12. The implementation of HTA findings needs to be monitored	+
13. HTA should be timely	+
Use of HTA in decision making	
14. HTA findings need to be communicated appropriately to different decision makers	++
15. Link between HTA findings and decision making processes needs to be transparent and clearly defined	+

Note. "+" signifies that the organization "supported" the principle in question in written guidelines or other form, regardless of whether they actually follow it. "++" means that the organization "implemented" the principle in published reports and decisions based on these reports demonstrate adoption of the specific principle. NICE = National Institute for Health and Clinical Excellence.

Social Values and End-of-Life Guidance

- NICE has always recognized that factors other than cost-effectiveness play a part in 'deliberative decision-making'.

(See Rawlins and Culyer , 2004: Citizens' Council, 2006)

- Question has always been one of how to operationalize this.
- The end-of-life guidance offers one potential solution.

NICE's Supplementary Guidance for 'End of Life' Therapies

- **If the therapy:**
 - is for a small patient population with life expectancy of less than 24 months;
 - where no equivalent therapy exists;
 - where the therapy adds three months or more to life expectancy,
- **Then:**
 - the QALYs gained should assume full quality of life in the added months;
 - in addition the Committee can consider that the QALYs gained should be weighted sufficiently high for the therapy to be approved given NICE's current threshold.

Issues Raised

- Is this the death of the QALY?
- Why stop with end of life? What about other socially valuable attributes?
- Are there better approaches:
 - weights for QALYs based on several factors
 - multi-criteria decision-making
 - different thresholds for different decisions?

Recognizing the Role of Innovation

- Lack of consideration of innovation is a long-standing industry criticism of NICE.
- Two important reports in the last year:
 - 'Kennedy' report, commission by NICE
 - Office of Life Sciences report, commissioned by the government.

Changes in NICE's Procedures

- Appraisal Committees can consider innovation to be a specific and identifiable benefit of the technology.
- Where the new technology is considered to represent a 'step change' the Committee has to demonstrate how it took this into account.
- Some technologies can be considered eligible for the '*Innovation Pass*' scheme.

Innovation Pass Scheme

- Arose mainly from the OLS report.
- Joint initiative between the government and NICE.
- Selective innovative medicines will be made available on the NHS for up to 3 years, to enable more evidence to be accumulated.

Issues Raised

- Funding for the scheme is 'only' £25 million.
- Technologies still have to appraised by the existing methods.
- Uncertainty about how the launch price of the technology will be determined.
- The scheme does handle issues like sequential innovation and dynamic efficiency.

And the Biggest Issue of All!

Since the US pays for most of the innovation in health care, what does the rest of the world do if the US ever manages to control its health care costs?

Enhancing Stakeholder Engagement

- NICE has always been in the forefront of stakeholder engagement.
- Nevertheless, the need for more stakeholder engagement was raised in the Parliamentary review of NICE.
- Recent changes include:
 - involvement of Evidence Review Groups in Scoping Workshops
 - sponsors can attend Appraisal Committee meetings
 - sponsor debriefing meetings introduced
 - program of early dialogue established

Program of Early Dialogue

- Initiated as a result of requests from manufacturers.
- Manufacturers can seek advice on evidence requirements, on a fee-paying basis.
- The main use is in determining clinical data requirements to support product reimbursement.

Issues Arising

- Advice is non-binding and confidential to the company.
- Currently NICE is the only body willing to provide written feedback to companies.
- What happens when NICE's requirements for clinical data are different from those of licensing agencies?
- Are we heading for some convergence between licensing and reimbursement requirements?

Developing Patient Access Schemes

- NICE has had several of these over the years, beginning with beta interferon for multiple sclerosis.
- NICE is now beginning to classify them (eg, into 'outcomes based' or 'financially based').
- A formal procedure is being set up and a *'Patient Access Scheme Liaison Unit'* established.

Recent Example of Risk-Sharing from the United Kingdom

Velcade (bortezomib) for Multiple Myeloma

- In the course of a NICE technology appraisal, an 'outcome guarantee' scheme was suggested by the manufacturer.
- The NHS will ensure that 'all suitable patients' will have access to the drug.
- In return, the manufacturer will refund treatment costs for patients who fail to respond.
- The details of the scheme, including the definition of 'clinical response' have now been agreed.

NICE Risk Sharing Examples

Uncertainty around clinical effect:

- b-IFN and glatiramer for multiple sclerosis – 2002
 - Prospective cohort – managed by DH...

NO uncertainty around clinical effect:

- Bortezomib for multiple myeloma – 2007
 - Money back guarantee based on response (M-protein)
- Ranibizumab for AMD – 2008
 - Dose capping scheme (<14 injections per eye)
- Erlotinib for SCLC – 2008
 - Cost capping scheme (same overall cost as docetaxel)
- Sunitinib for advanced RCC – 2009 DRAFT
 - First time EOL guidance informed decision
 - 1st cycle of treatment free to NHS patients
- Lenalidomide for multiple myeloma – 2009 DRAFT
 - Dose capping scheme (<26 cycles/2yrs)

Source: Chalkidou (2009)

Issues Arising

- In the design of the schemes, the devil is in the detail.
- Some of the schemes are merely price cuts through the back door.
- Is NICE morphing into a price negotiation body?

Conclusions

- NICE has achieved a considerable amount in its first ten years.
- It continues to evolve and has lots of unfinished business.
- Like all such bodies it is the product of the healthcare system in which it is based.
- It offers important lessons to other jurisdictions, but is not simply transportable to other settings.
