



Health technology assessment

Summary of introduction workshop in 2013



Overall aim

To spend money wisely

Three questions

- Does it work?
- How well does it work?
- What does it cost?

Does it work?

- Critical appraisal
- Effect size

A choice... and a decision

- Drug A

- Costs £10 000

- Prolongs life by 2 years

- Drug B

- Costs £100 000

- Prolongs life by 50 years



Quantity and quality

- Prolongs life
- Improves quality of life



Quality of life

- Reduction in disability
- Health state

Quality adjustment

- 5 dimensions:
 - Mobility
 - Self care
 - 'Usual activities'
- Pain
- Anxiety or depression

Health state

State A

- Severe pain
- Moderate depression
- No mobility problems
- Some problems with usual activity
- No problem with self care
- 0.16

State B

- No pain
- Moderate depression
- Some mobility problem
- Severe problems with usual activity
- No problem with self care
- 0.42

- Growth hormone £100 000
- Gain in quality – 0.1
... for 50 years = 5 QALY
- £20 000 per QALY

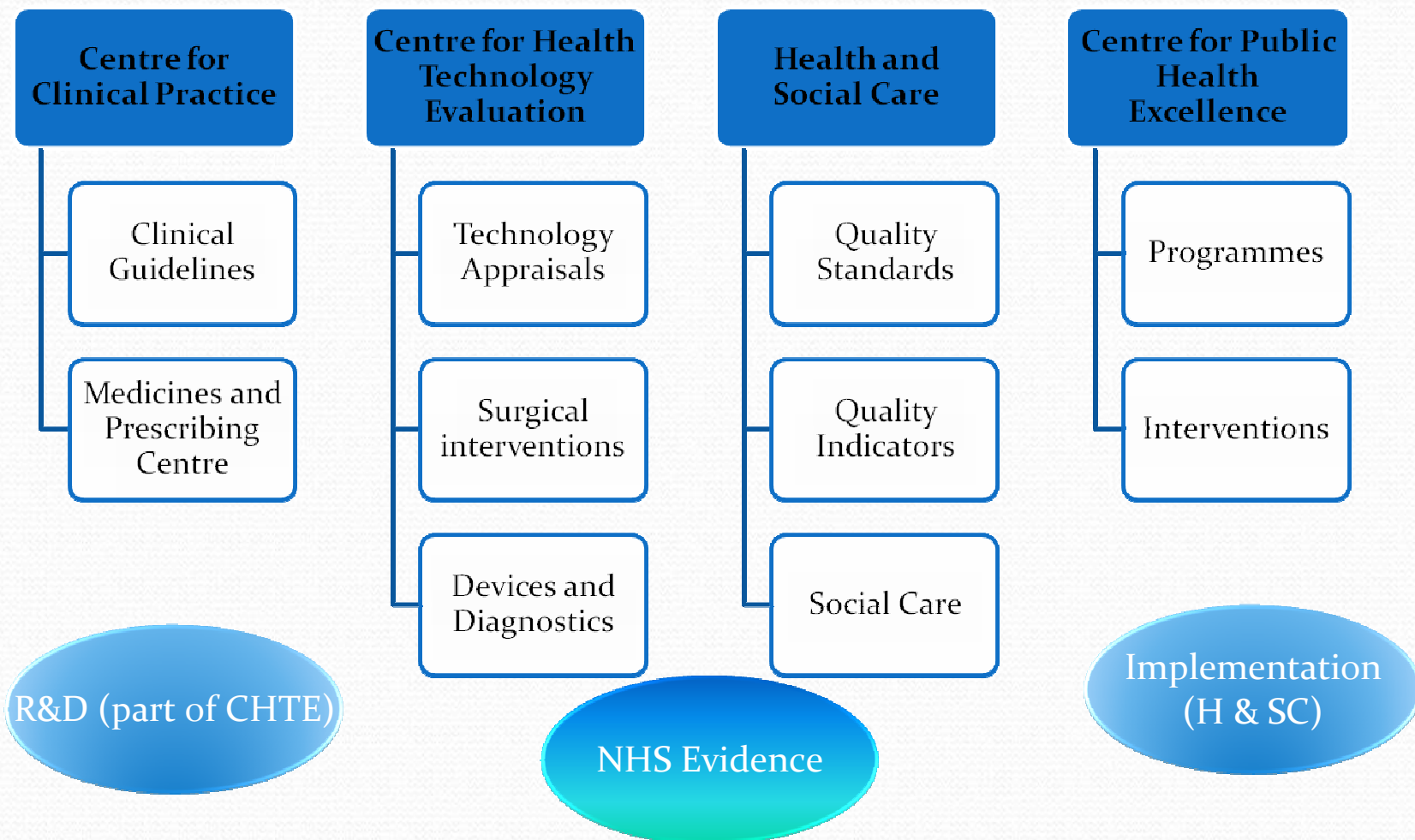
The image features a solid blue background with a gradient. At the top, there are several wavy, horizontal lines in shades of light blue and cyan, creating a sense of movement or a horizon. The word "NICE" is written in a bold, white, sans-serif font, positioned on the left side of the image.

NICE

- National Institute for Health and Care Excellence
- 303 appraisals since March 2000
- Includes orphan drugs
- Separate stream for ultra orphan
- Usually 'no' if >£30 000 per QALY

Structure and activities

Patient and
public
involvement
(H & SC)



NICE technology appraisals

- Medicines
- Devices (e.g. hearing aids or inhalers)
- Tests used to identify diseases
- Procedures (e.g. removal of wisdom teeth)
- Health promotion (e.g. ways of helping people with diabetes manage their condition).

- ‘Accountability for reasonableness’
A4R
- Transparent – all documents public
- Allow appeals
- Update if new information

Right to Appeal

- **Patients and Carers**
- **Professionals**
- **Industry**
- **Government**
- **Payers**



-
- The HTA report goes to a committee
 - Committee judgement
 - Lay input
 - Social value judgements
 - End of life

End of life treatments

- The treatment is indicated for patients with a short life expectancy, normally less than 24 months

AND

- There is sufficient evidence to indicate that the treatment offers an extension to life, normally of at least an additional 3 months, compared to current NHS treatment

AND

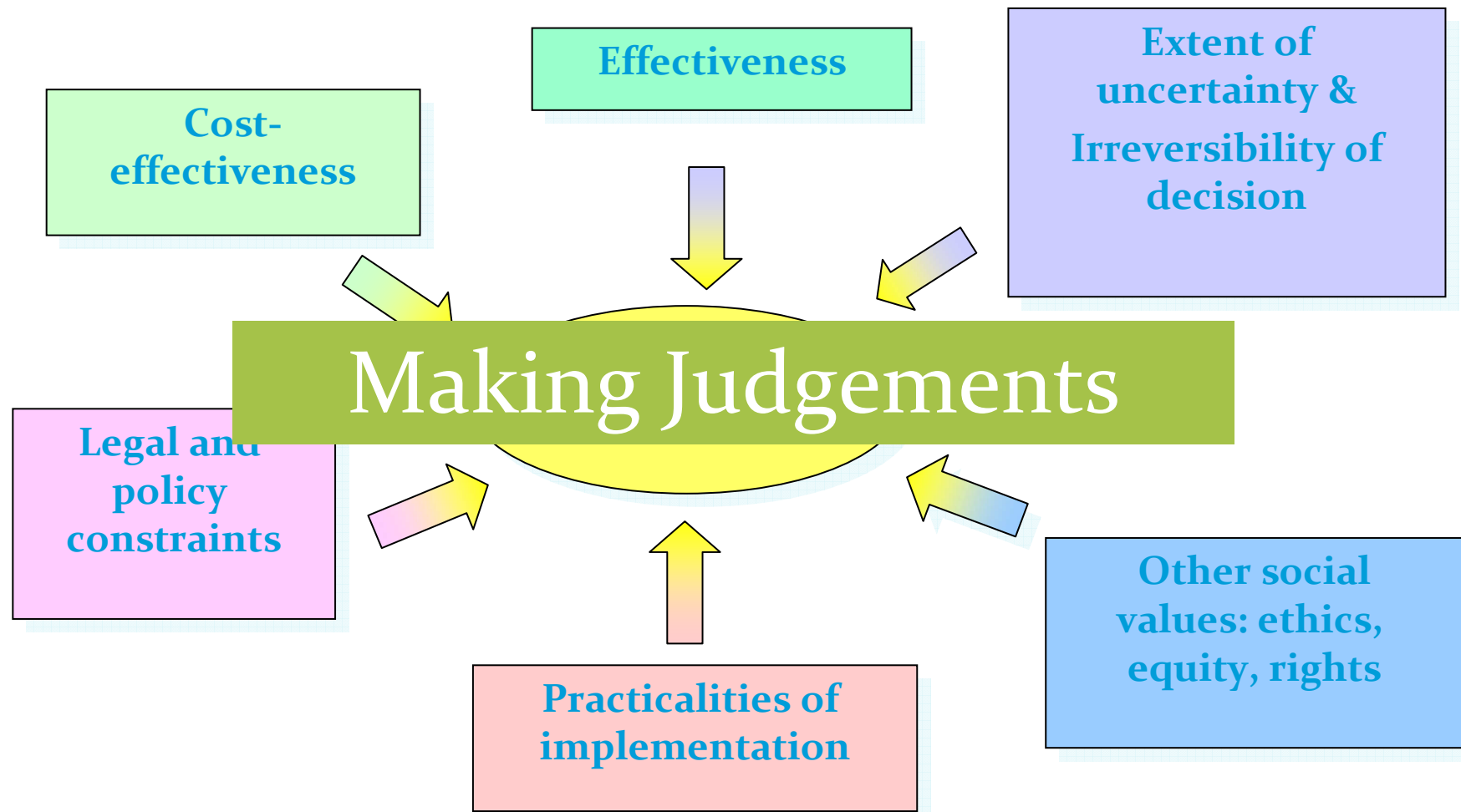
- The treatment is licensed or otherwise indicated, for small patient populations

- Plus

- Estimates of extensions to life are robust ...
 - Assumptions in the economic model are plausible and robust



Factors involved in NICE decision making



Technology appraisals

Guidance on the use of new and existing medicines, treatments and procedures within the NHS

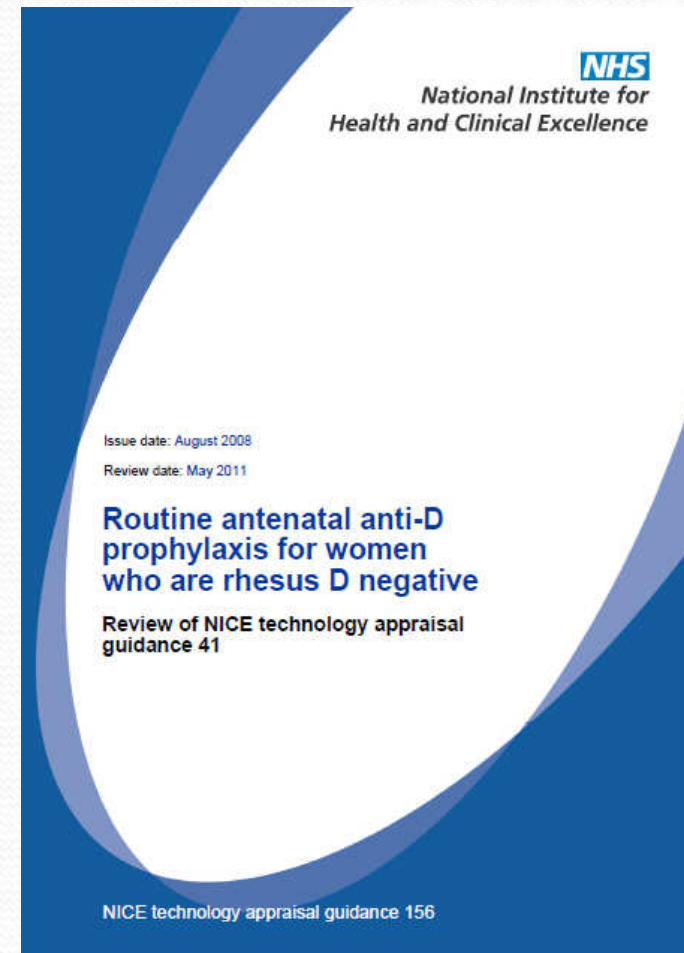
Two types of appraisals:

Multiple Technology Appraisal (MTA)

Single Technology Appraisal (STA)

- Independent academic groups carry out systematic review and develop economic model (MTA) [60 weeks]
- Critique the evidence submitted by manufacturer (STA) [30-43 weeks]
- 4 standing Committees (up to 33 members each)

Recommendations finances for implemented to be made within 3 months unless clear reason for delay



Economic Evaluation

Overarching principles:

1. Economic perspective

- Health system
- “time costs” of carers when care might otherwise have been provided by the NHS/PS

2. Cost effectiveness (cost / QALY)

- Not affordability or budgetary impact



NICE reference case

- The 'decision problem'
- Comparators
- Perspective on costs and health outcomes
- Type of economic evaluation
- Synthesis of evidence on outcomes
- Measure of health effects
- Source of data for the measurement of HRQL
- Source of preference data for the valuation of changes in HRQL
- Discount rate
- Equity weighting

Summary of grounds cited for appeals

1 March 2000 to 31 October 2012	Number of appeals	
Ground 1: The Institute has failed to act fairly.	63	(38%)
Ground 2: The Institute has formulated guidance which cannot reasonably be justified in the light of the evidence submitted.	71	(43%)
Ground 3: NICE has exceeded its powers	31	(19%)
Total	165	

The percentages in the table may not add up to 100% because appeals may be made on multiple grounds.

There are three possible grounds for appeal:

^a Ground 1 - NICE has failed to act fairly and in accordance with its published procedures as set out in the '[Guide to the technology appraisal process](#)'

^b Ground 2 - NICE has prepared a Final Appraisal Determination that is perverse in the light of the evidence submitted

^c Ground 3 - NICE has exceeded its powers (that is, NICE has acted outside its remit or unlawfully in some other way)



Uncertainty and NICE Appraisals

- Ubiquitous
- Note values of:
 - **Transparency**: methods, evidence base and decisions are public
 - **Scientific basis**: peer review and methods development
- Context is evidence *synthesis* – bringing things together to make sense and reach *reasonable* conclusion

Elements in an assessment

- Population, Intervention, Comparator and Outcome (scope of the assessment)
- Technology *effectiveness*
- Value of outcomes (QALY issue)
- Technology cost / savings
- Timing of events
- Perspective of the analysis (societal or NHS?)



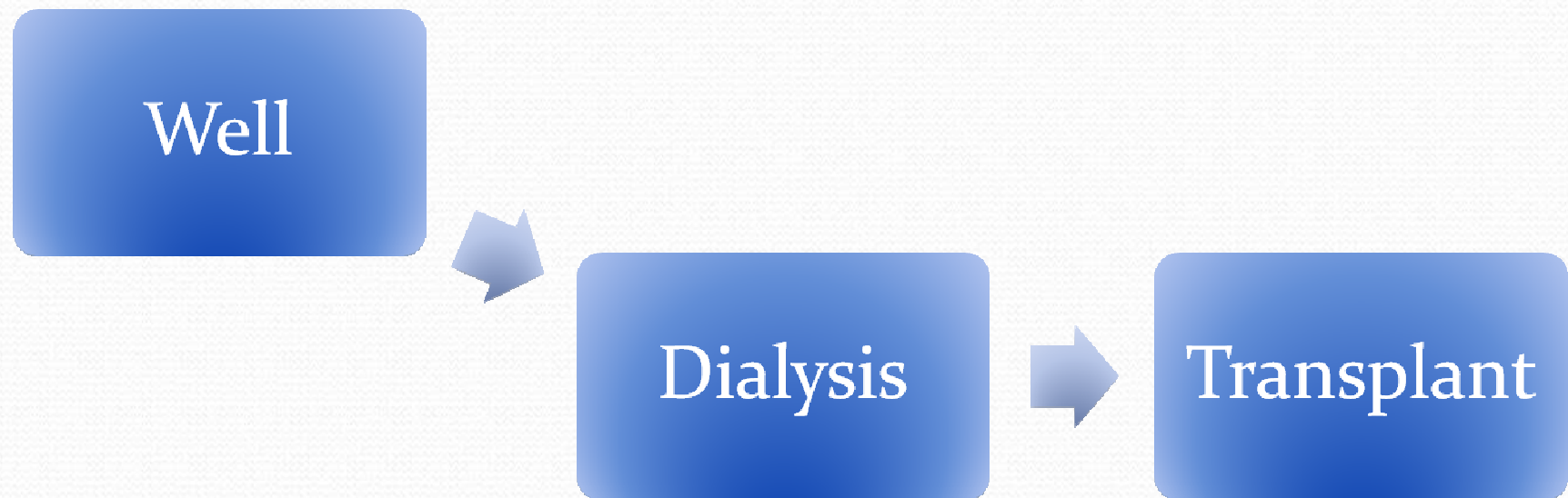
Systematic review

- Find the “right stuff” (least biased)
 - Search hard, use filters
- Check if you should believe it
 - Structured appraisal depending on design
- Work out what it means
 - Synthesis (narrative or meta-analytic)
- Consider what implications for practice or policy
 - Some kind of “model” of what impact the technology might have (on individual or population) ... modelling

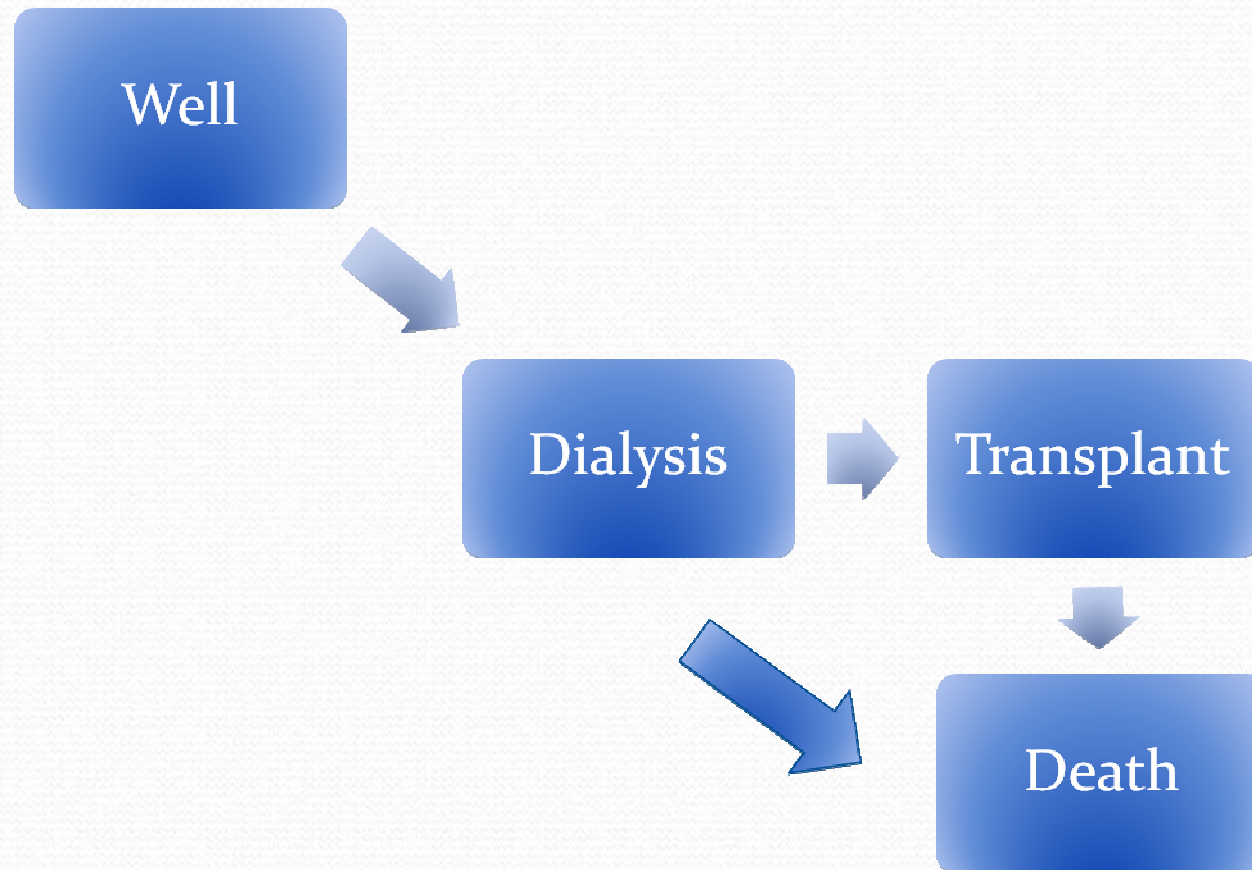
Modelling disease states

Transition probabilities

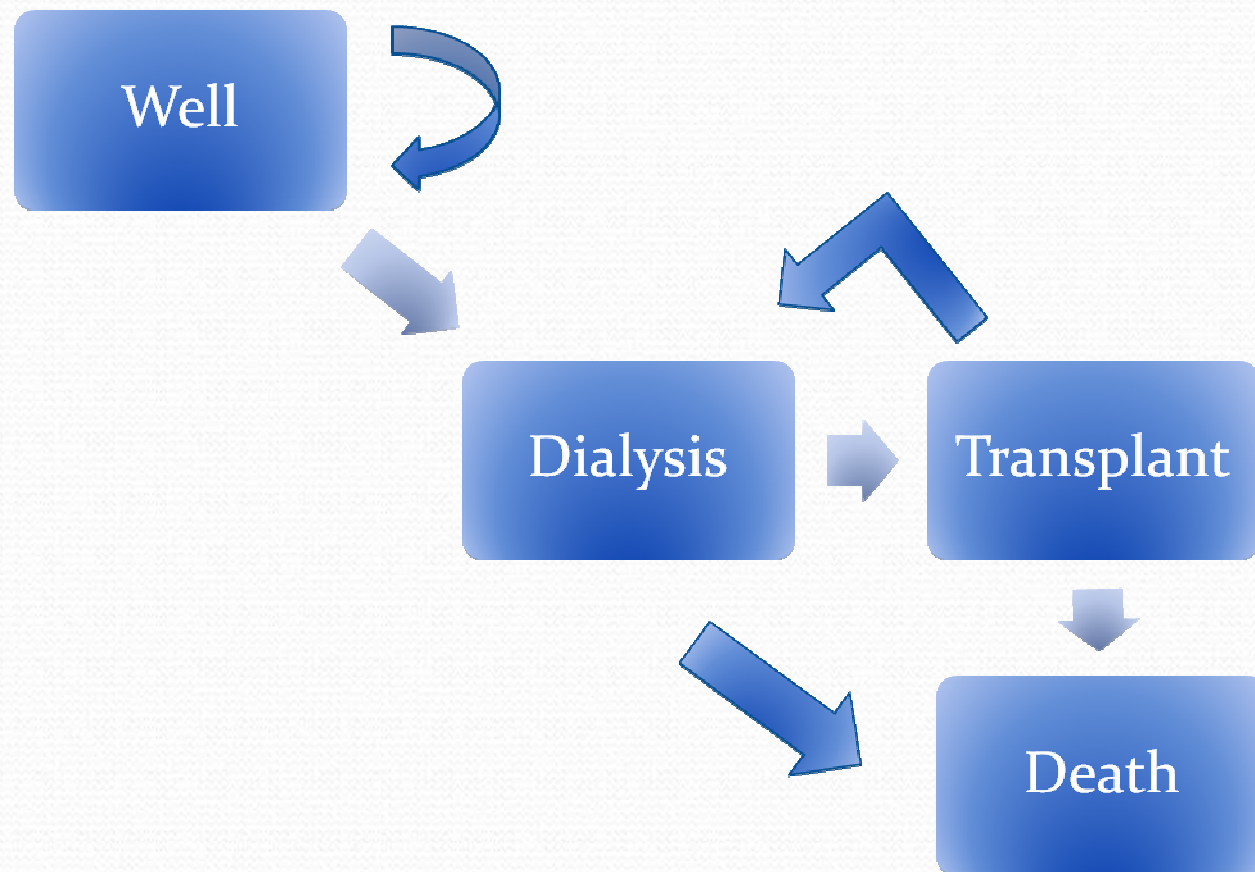
Disease states



Transitions



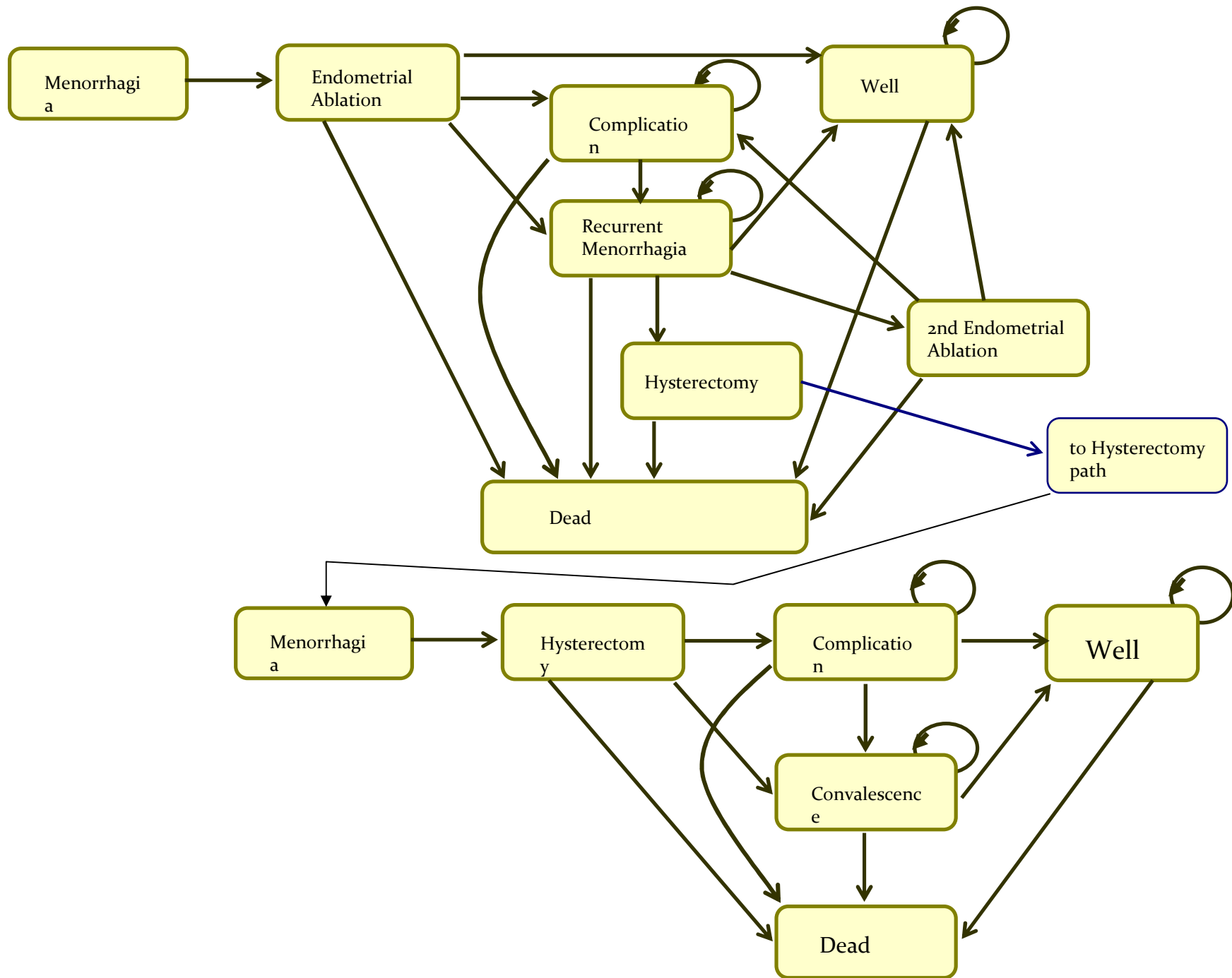
A 6month cycle



The effectiveness and cost-effectiveness of microwave and thermal balloon endometrial ablation for heavy menstrual bleeding: a systematic review and economic modelling

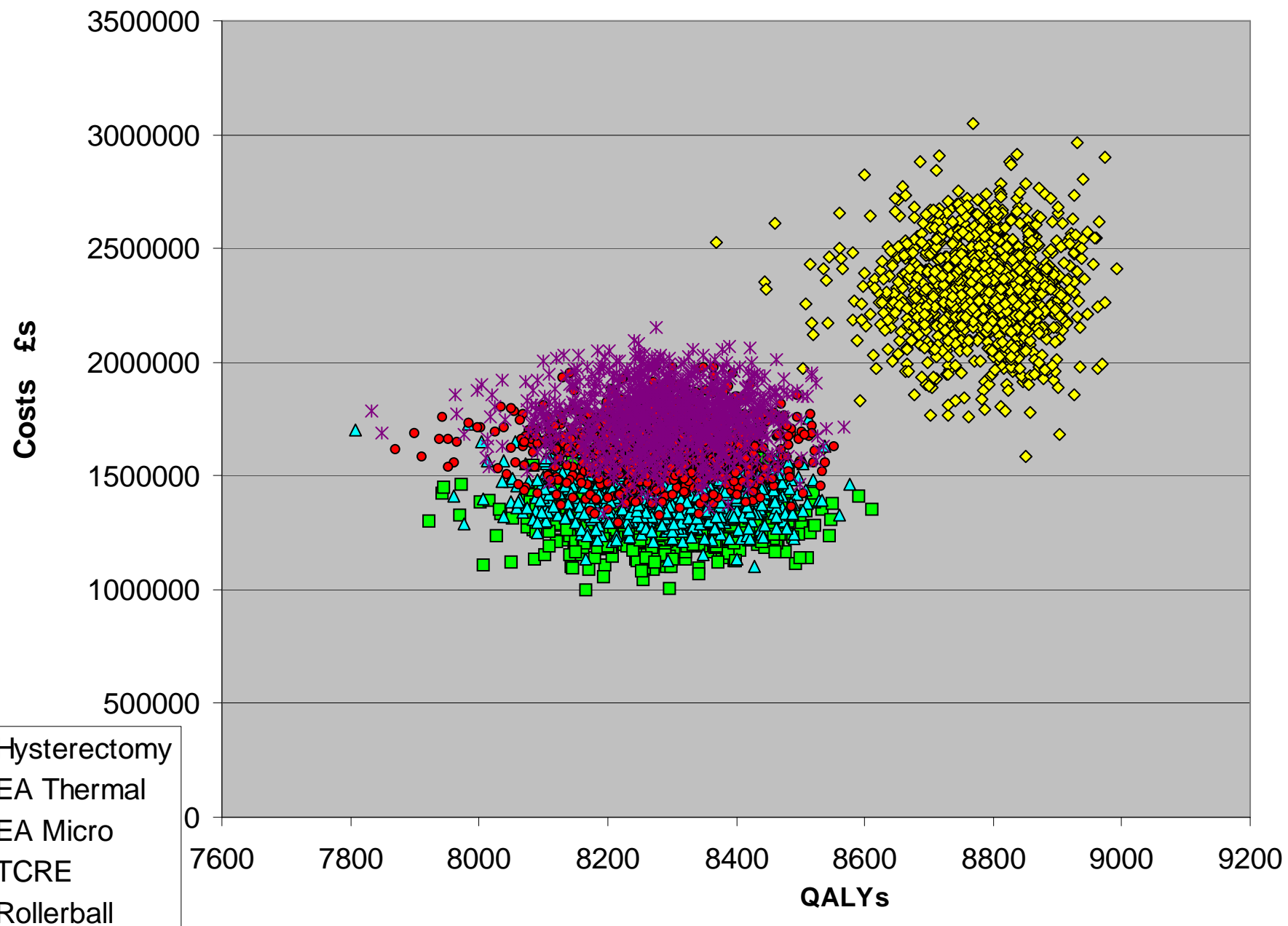
R Garside, K Stein, K Wyatt, A Round
and A Price





Point estimates and probabilities

- ‘The cost per QALY is £25 000’
- Cost – varies from patient to patient
- Benefit – varies from patient to patient
- Need an estimated RANGE for cost and benefit



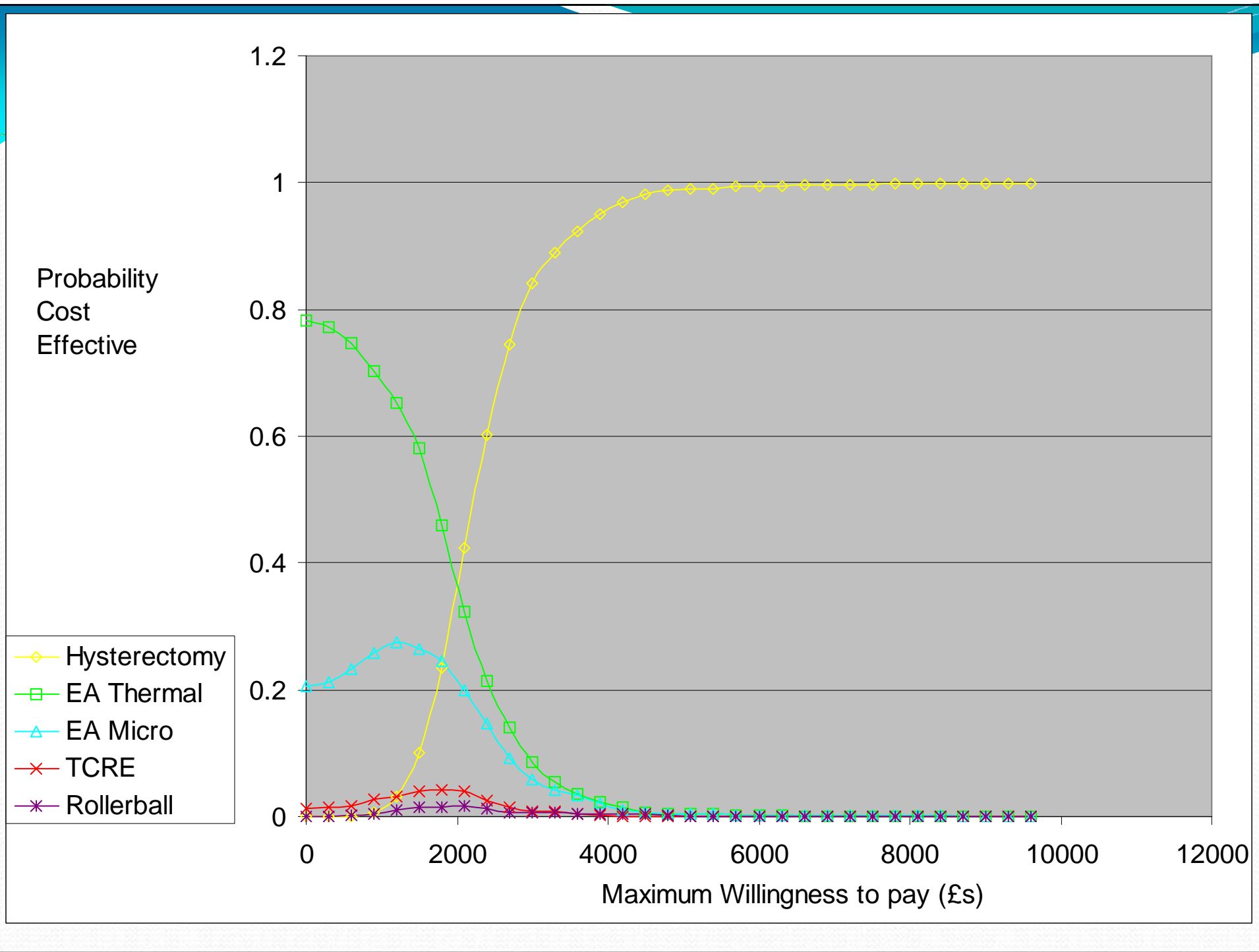


Table 1

Application of 'special circumstances' in the appraisal of some products with incremental cost-effectiveness above £30 000 per quality adjusted life year

Topic	ICER ('000s)	Severity	End of life*	Stakeholder persuasion	Significant innovation	Disadvantaged population	Children
Riluzole (motor neurone disease)	38–42	✓	✓	✓			
Trastuzumab (advanced breast cancer)	37.5	✓			✓		
Imatinib (chronic myeloid leukaemia)	36–65	✓			✓		
Imatinib (gastrointestinal stromal tumour)		✓	✓		✓		
Pemetrexed (malignant mesothelioma)	34.5	✓	✓			✓	
Ranizumab (age-related macular degeneration)	>>30			✓	✓		
Omalizumab (severe asthma)	>30	✓		✓	✓		
Sunitinib (advanced renal cancer)	50	✓	✓	✓	✓		
Lenalidomide (multiple myeloma)	43	✓	✓		✓		
Somatotropin (growth hormone deficiency)	n/a			✓	✓		✓
Chronic subcutaneous insulin infusion (childhood Type 1 diabetes)	n/a			✓			✓

*End-of-life considerations have only been explicitly taken into account since January 2009 on the basis of supplementary advice from the Institute to the Appraisals Committee.
ICER, incremental cost-effectiveness ratio (£ per quality-adjusted life year).

Rawlins, Barnett, Stevens Br J Clin Pharmacol 2010



Other ways of improving access...

- NOW

- “Patient Access Schemes”...
 - Getting discounts without altering list price

- FUTURE

- “Value-based pricing”
 - products which improved the health status of patients significantly would be priced more highly than those drugs that are comparatively less effective

