Who is involved in making NICE guidance recommendations and what evidence do they look at?

Lizzie Thomas, Senior Public Involvement Adviser
Who decides what NICE will recommend?

1. Specialist staff employed by NICE
2. The Department of Health
3. NICE employed administration staff
4. NHS England
5. Clinical Commissioning Groups
6. NHS finance managers
7. Patients
8. Independent committees of NICE staff & experts
9. Independent committees of experts
Who decides what NICE will recommend?

**Independent committees**

- Chair
- At least 2 lay members
- Health and other professionals (specialists and generalists)

Sometimes
- Technical experts e.g. health economist

- 2 types: standing committees and topic specific groups
- Staff provide technical and administrative support
Evidence informing committee’s work

- Reviews of research evidence (all NICE guidance)
- Stakeholder consultation (all NICE guidance)

- Grey literature and unpublished data
- Economic modelling
- Manufacturers submissions
- Expert testimony (patient and professional)
- Occasional additional consultation or fieldwork with practitioners and patients

NICE recommendations based on **best available evidence**
The right evidence type for the question

The question dictates the most appropriate study design:

- 'What is the cause of this disease?' Cohort, case-controlled study
- ‘What does it feel like?’, ‘What is important to you?’ or ‘What is your experience of care?’ Qualitative research
- 'What is the most clinically effective therapy?' Randomised controlled trial (RCT)
- ‘What works best in diagnosing the condition?’ Observational study or RCT

Includes systematic reviews of studies when available
The nature of evidence

- Patient evidence
  - High quality patient care
- Clinical evidence
- Economic evidence

Acknowledgement: Dr Sophie Staniszewska, RCN Research Institute, University of Warwick
Patient evidence
The value of patient evidence

What insights does patient evidence offer us?

- Personal impact of living with a condition and experience of care
- People’s preferences and values
- Outcomes that patients want from treatment or care
- Impact of treatment or care on outcome, symptoms, physical and social functioning, quality of life
- Risks, benefits and acceptability of a treatment or service
- Equality issues and considerations for specific sub-groups
Evidence from experience of care

High dose rate brachytherapy for cervical carcinoma

People who self-harm

Age-related Macular Degeneration

Psoriasis

Kidney dialysis
Patient perspectives: impact and challenges

Examples of positive influence of patient evidence on:

- Scoping and review questions
- Evidence reviews – how interpreted
- Guidance recommendations
- Research recommendations

Challenges

- Ensuring patient voices are heard
- The weighting of patient evidence
- Synthesising with clinical and economic evidence
Health (Care) Economics
Ross Maconachie

Wednesday 28th Sept 2016
Why Consider Health Economics?

Opportunity Cost

• If the NHS spends more on one thing, it has to do less of something else (on the margin)

• Could we do more good by spending money in other ways?

• The ‘opportunity cost’ is the value of the best alternative use of resources
Which Mobile Phone Contract Do You Recommend?

Option A
- Unlimited minutes
- Unlimited texts
- 4 GB of data
- £25 per month

Option B
- 500 minutes
- 500 texts
- 1 GB of data
- £20 per month

Thinking about cost-effectiveness and opportunity costs – which contract do you pick??
There is more information that we would need here, ideally:

• What is the budget/who is paying? This is our threshold
• Who are we making the decision for (perspective)?
• How long for?
• What benefits will the person get?
• What is the opportunity cost? → the value of the best alternative forgone.
Economic Evaluation
What to Consider?
Economic Evaluation

• Cost-effectiveness, not cost (or resource) impact
• “... the comparative analysis of alternative courses of action in terms of both their costs and consequences.”
  – Drummond, Stoddard & Torrance, 1987

Costs
• Value of extra resources used (loss to other patients)

Current Treatment

New Treatment

Consequences/Outcome
• Value of health gain for this patient group
What Health Outcomes are Important?

What we want to know….
• How much the intervention improves the health of the people involved?
• What measure to use…..or to develop
• What outcomes/factors should we be looking at?

Types of Outcome…..
• Cure? ‘Progression-free’?
• Improved measurement? (e.g. growth; reduced blood pressure
• Reduction of risk? (e.g. pre-term birth; falls in older people)
• Improved ‘quality of life’?
• Additional years (or months) of life?

Tools to measure quality of life (QoL) were developed and are available in a wide range of areas of health.

At one point or another you will come across one, if not all of these outcomes – there is no one perfect outcome. However……
Examples of Measures

Generic (disease non-specific) – how we get a QALY
• EQ-5D – EuroQol 5 Dimension
• SF-6D and SF-36 – Short-Form
• HUI – Health Utilities Index

Disease Specific
• AQLQ- Asthma Quality of Life Questionnaire
• MSQOL - Migraine Specific Quality of Life
• EORTC QLQ-C30 -European Organization for Research and Treatment of Cancer Quality of Life Questionnaire

Add-On Tools
EQ-5D+C – add on for cognitive factors
# Describing Health State Using EQ-5D

<table>
<thead>
<tr>
<th>Mobility</th>
<th>I have no problems in walking about</th>
<th>I have some problems in walking about</th>
<th>I am confined to bed</th>
</tr>
</thead>
<tbody>
<tr>
<td>Self-care</td>
<td>I have no problems with self care</td>
<td>I have some problems washing or dressing myself</td>
<td>I am unable to wash or dress myself</td>
</tr>
<tr>
<td>Usual activities</td>
<td>I have no problems with performing my usual activities</td>
<td>I have some problems with performing my usual activities</td>
<td>I am unable to perform my usual activities</td>
</tr>
<tr>
<td>Pain/discomfort</td>
<td>I have no pain or discomfort</td>
<td>I have moderate pain or discomfort</td>
<td>I have extreme pain or discomfort</td>
</tr>
<tr>
<td>Anxiety/depression</td>
<td>I am not anxious or depressed</td>
<td>I am moderately anxious or depressed</td>
<td>I am extremely anxious or depressed</td>
</tr>
</tbody>
</table>
Valuing Health States Using EQ-5D

- EQ-5D defines 243 possible states
- Values of states elicited from UK general population (n=3,395)
- Has been developed in different countries for that specific population
- Mean values can be used in economic evaluations
- Values elicited using ‘time trade-off’ – so 1 year in perfect health = 2 years in 0.5 health
EQ-5D Exercise

1. Read the Vignette describing a condition
2. Fill in the EQ-5D form imagining you are that patient
3. Use the formula to calculate your EQ-5D score
4. Discuss

(NB: There is no ‘correct’ answer to this exercise)
Quality Adjusted Life Years - QALY

• What is a QALY?
  – QALYs combine both quantity and health-related quality of life (QoL) into a single measure of health gain
  – Use a self reported preference-based questionnaire score (like EQ-5D or SF-36)
  – QoL scores (utilities) should reflect people’s preferences over health
  – Utilities usually scored with ‘perfect health’=1 and death=0
Quality Adjusted Life Years

• Why use QALYs?
  – Can weigh up net effect of treatment for patients
  – Survival vs QoL (e.g. for cancer chemotherapy)
  – Long-term QoL for chronic & recurrent conditions (e.g. arthritis)
  – Benefits versus harms (e.g. COX II inhibitors)
  – Allows broader comparisons between patient groups

The QALY is the preferred measure of NICE when conducting cost-effectiveness analysis (mostly in clinical and technology appraisals). However, not always possible for social care and public health so look to other methods.
"A QALY is a QALY is a QALY"

• Usual value judgements used to calculate QALYs:
  – Allows the effectiveness of different technologies for different people to be compared

• 1 QALY:
  – One year of ‘perfectly’ health life for one person
  – OR two years of life with QoL of 0.5 for one person
  – OR one year of life with QoL of 0.5 each for two people

**BUT** – they do not incorporate an age weigh function – so 1 QALY at age 86 = 1 QALY at age 3
Quality Adjusted Life Year

- Health-related quality of life
- Length of life (years)
- QALYs gained

Current treatment

New treatment

Initial QALY loss due to side effects

QALYs gained
Economic + Cost-Effectiveness Analysis
### Types of Economic Evaluation

<table>
<thead>
<tr>
<th>Type of analysis</th>
<th>Value of resources</th>
<th>Value of health gain</th>
</tr>
</thead>
<tbody>
<tr>
<td>Cost / Cost impact</td>
<td>£</td>
<td>None</td>
</tr>
<tr>
<td>Cost-effectiveness</td>
<td>£</td>
<td>Single indicator: Weight loss (kg), blood glucose control (HbA1c), deaths averted, life years saved...</td>
</tr>
<tr>
<td>Cost-utility</td>
<td>£</td>
<td><strong>Combined index:</strong> Quality Adjusted Life Years (QALY)</td>
</tr>
<tr>
<td>Cost-benefit</td>
<td>£</td>
<td>Monetary value: Willingness to pay (£)</td>
</tr>
</tbody>
</table>

⚠️ **Warning** – ‘cost-effectiveness’ often used as general term for any analysis with non-monetary measure of health gain.
Assessing Cost-Effectiveness

- Treatment cost-effective in shaded region
- CE threshold ~£20-30K
- New treatment dominated
- New treatment dominated
- Low extra cost High QALY gain
- High extra cost Low QALY gain

ΔCost (£)

£/QALY

ΔEffect (QALYs)
The Threshold

• If one intervention does not ‘dominate’ then we use a threshold of £20-30k QALY to decide

• Not set in stone – other considerations:-
  – Health gain not captured in the analysis
  – End of life criteria
  – Significant innovation
  – Certainty around the estimate

• Theoretical basis - £20k/Q represents maximum WTP in a perfectly allocatively efficient NHS
Modelling – Why Do It?

- Trials are of limited duration and so may not capture all costs and benefits.
- A single trial may not capture all the relevant information:
  - Not all the relevant outcomes (e.g., adverse effects, quality of life, costs).
  - Not all the relevant comparators [there may be several relevant options that are not feasible to examine concurrently in one single trial].
- More than one study may address the clinical decision.
- Trials often report intermediate outcomes:
  - E.g., Blood pressure reduction.
- An intervention may have both positive and negative effects.
- There may not be a trial – social care and public health in particular.
- Trials can be very expensive.
- It may be unethical to carry out a trial in a particular area.
What is a model

- A mathematical structure used to combines different sources of data

**Test accuracy**
- Sensitivity/specifcality

**Treatment effects**
- Survival, health status

**Resource use**
- GP visits, inpatient stays...

**Unit costs**
- e.g. £ per GP visit

**Preferences**
- QoL weights

**Epidemiology**
- Baseline risks, sub-groups

**Cost Effectiveness**
- £/QALY
Cost-Effectiveness in NICE Guidelines

- Principles set out in NICE’s Social Value Judgements:

**Principle 2** - “Those developing clinical guidelines, technology appraisals or public health guidance *must take into account the relative costs and benefits of interventions* (their ‘cost effectiveness’) when deciding whether or not to recommend them.”

**BUT**

**Principle 3** - “Decisions about whether to recommend interventions should not be based on *evidence of their relative costs and benefits alone*. NICE must consider other factors when developing its guidance, including the need to distribute health resources in the fairest way within society as a whole.”
Any Questions?