Ensuring that the patient voice is heard: Experience from the UK

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Ensuring transparency

• NICE actively seek the widest possible input on their Appraisal Consultation Documents from clinical experts, patient interest groups and society through their open publication of their preliminary findings on the web.

• The NICE technical team will summarize all responses to the request for consultation for discussion at the next meeting which aims to produce the Final Appraisal Determination (FAD).

• Such Extensive consultation adds considerable time to the NICE review process but provides the widest possible opportunity for all interested parties to contribute to the quality of the decision making process.
The contributions of stakeholders

• Manufacturers provide
  – All relevant papers, published or not
  – Model or other method of estimating cost-effectiveness
  – Analysis and discussion

• Professional groups provide
  – Scientific expertise and professional experience

• Patient groups provide
  – Direct evidence of the condition and treatment implications
The Pharmaceutical Industry as a stakeholder

- Scottish Medicines Committee – 3 SMC members from industry
  - Ensure due process and fairness
  - Act as informal channel of (2-way) communication
  - The first port of call for ‘unhappy’ companies

- NICE – more limited industry involvement
  - The need to be unambiguously perceived as being fair and independent
  - Strictly controlled channels of communication
The Political process as a stakeholder: Cancer Drug Fund

- Politicians criticised NICE over restricting access to cancer drugs
- New government creates ring fenced £200M fund to support the use of cancer drugs that had been rejected by NICE – recently extended with a further £160M
  - Leading to variations in local availability of cancer drugs
  - Undermines NICE principle of a single cost-effectiveness threshold to optimise the drug budget.
Stakeholders aims will conflict!!!

• The UK consumes 3% of the world’s drugs but receives around 9% of global pharmaceutical industry investment.

• Trade stakeholders within the UK government support policies to achieve high prices and volumes for new drugs.

• Health stakeholders within the UK government support policies to obtain low prices for new drugs to achieve value for money for the NHS.
‘Society’ and the ‘patient’ as stakeholders in HTA

• We need to address two questions......

• Question 1 – How can we ensure that the patient’s voice is effectively heard in HTA decision making?
• Question 2 – How can we ensure that society’s priorities underpin our decision making?
Empowering patients at NICE

• Every NICE Appraisal committee consists of lay members whose specific role is to reflect the ‘patient perspective’ in Appraisals
• Every ‘lead team’ clinical presentation contains a segment on the patient perspective presented by one of the lay members
• Every Appraisal committee meeting has clinicians and patient representatives with direct experience of the disease under evaluation.
Patient involvement at NICE

Stage 1
Scoping the questions to be addressed

Stage 2
Evaluating the health technology

Scoping usually occurs:
1. Before a licence has been granted for a technology
2. Before the topic has been referred

Guidance development is usually:
1. After referral
2. As close to issue of licence as possible
   (the licence has to be issued before the appraisal consultation can begin, after the committee meeting)
The patient voice from beginning...

Patient organisations can:
1. comment in writing on the draft
   - remit
   - scope
   - matrix (stakeholder list)
2. attend scoping workshop

Key
Active involvement of patient organisations

Scoping consultation

Final scope produced
Patient experts...
Attend committee meetings.
Provide evidence to Committee
Comment on ACD and Final Appraisal Determination
Guidance Published
NICE: summary of patient involvement

• **Three lay people** on each appraisal committee

• **National, condition relevant patient groups**:
  - attend scoping workshops
  - comment on draft documents
  - provide written submissions of evidence
  - nominate patient experts
  - Submit an appeal

• **Individual patients/carers** attend as patient experts, provide personal statements and comment on draft recommendations
Patient evidence: collection and analysis

- Obtain **oral evidence** directly from patients to help the appraisal committee understand the topic and issues
- Obtain **written evidence** from patient groups using a Standard template (blank version available on the NICE website)
  - These represent an essential part of the formal evidence-base for each topic
  - This evidence is supported by the presence of three ‘patient advocates’ who sit on the committee
The value of Patient Submissions

• Provides ‘hot’ evidence concerning the real world burden imposed on patients by the disease

• Provides evidence about the real world benefits that will arise to patients as a consequence of being able to access the drug

• Enables the views of Carers to be represented and incorporated into the evaluation
The value of the patient perspective

1. **Having the condition**, or caring for someone with the condition
2. **Receiving care** for the condition in the healthcare system
3. **Having specific treatments**
4. **Outcomes of treatments** that are important for patients and carers
5. **Acceptability** of different treatments
6. **Preferences** for different treatments
7. **Expectations** about the risks and benefits of the technology
Case study: Lenalidomide for myelodysplastic syndrome

- Drug is taken orally, therefore non-invasive and simple to administer.
- Can vastly improve quality of life, by removing constant tiredness, shortness of breath, and susceptibility to infections.
- Enables a return to normal work, and increased levels of physical exercise and daily activities, contributing to a vastly improved outlook on the future and much more positive emotional well-being.
- Perceived as a disease modifying drug and treatment option, compared with the current palliative treatment of frequent blood transfusions with the associated risks, complications, time and costs.
- Generally well tolerated, with very few adverse reactions reported.
Beware patients as advocates!

• Invited Patient representatives inevitably advocate on behalf of their therapeutic area.
• Their advocacy role means that they are not expected to take into account wider concepts such as opportunity cost.
• Committee members frequently perceive patient organisations as being an ‘indirect voice’ for the drug industry.
‘Society’ as a stakeholder

• The ‘values’ underpinning HTA decision making should reflect social value judgements held by society with regard to distributional and procedural justice.

• Central to this objective is the work of the ‘Citizens Council’ a group of 30 members of the general public chosen independently of NICE to establish such values.
NICE Citizens Council
Council members include:

- An electrician
- A clerical officer
- A store assistant
- A retired security officer
- A housewife
- A secretary
- A teacher
- A taxi driver
Case study: End of Life care

- NICE citizen juries emphasised that NICE should give special consideration to treatments at end of life... “which may be life-extending for patients with short life expectancy, and which are licensed for indications affecting small numbers of patients with incurable illnesses.”

- Under specific circumstances EoL Care can have up to a 66% additional weight attached to their outcomes. In effect, accept a higher cost/QALY threshold (£50000 rather than £30000).

- This guidance was based on the belief that patients value life more highly when their remaining life expectancy is short.
Empowering stakeholders – the issues

- How do we effectively incorporate evidence on patient views, experiences and preferences into decisions?
- Do Quality of life measures utilised by clinicians adequately capture issues of greatest importance to patients?
- What weight should HTA agencies place on evidence obtained from patient submissions and patient experts?
- How do we ‘interpret’ the technical language and modelling discussed in Appraisal committees to make them more accessible to enable patient representatives to effectively participate?
Conclusion

• Whether to inform individual assessments or ensure consistency with societal values the voice of the patient is crucial.
• However patients have an advocacy role on behalf of their particular disease which restricts their focus.
• HTA has to place the demands for resources by each disease in a wider context.
• Decisions of HTA groups have to reflect the values of society and also ensure that all patient groups are evaluated on a ‘level playing field’.
More information

Public homepage
www.nice.org.uk/getinvolved/patientsandpublic

PPI Policy
www.nice.org.uk/getinvolved/patientandpublicinvolvement/patientandpublicinvolvementpolicy/patient_and_public_involvement_policy.jsp

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