Can patient involvement in early dialogues increase the value of the advice given?

Moderator: Nicholas Brooke, PARADIGM & PFMD
Speakers: Neil Bertelsen, HTAi
Heidi Livingstone, NICE
Maggie Galbraith, HAS / EUnetHTA
PARADIGM

- A Distinct Voice In the Patient Engagement Landscape

Mission
Contribute to a sustainable framework that enables meaningful patient engagement (PE) and demonstrates ‘return on engagement’ for all players

Objectives
Develop processes and tools for these three points in the medicine lifecycle
Develop a sustainability roadmap for patient engagement

Multi-stakeholder collaboration
- to drive meaningful and systematic patient engagement
A Virtuous Development Process

Needs
- Survey to understand stakeholder needs
- Focus groups
- Delphi methodology

Practices
- Identification of PE practices
- Gap analysis

Tools & metrics
- Recommendations
- Process & Practices
- Evaluation with metrics

Sustainability
- Sustainability of PE
- Toolkit uptake
- Institutionalisation of recommendations

Internal and external communication and engagement

Project Management – to promote effective partner collaboration for timely delivery of high quality outcomes

Collaborative Initiatives Dedicated to Patient Engagement

Global Focus
Patient Engagement Guidance & Tools
Full Medicine Lifecycle
No Time Boundary

European Focus
Patient Engagement Guidance & Tools
Time Points: Research Prioritization; Clinical Trials; Early Dialogue Regulatory & HTA Bodies
30 Months Duration
http://patientfocusedmedicine.org/our-partners/

European Focus
Patient Capability Building
Full Medicine Lifecycle
No Time Boundary
http://imi-paradigm.eu/project-partners/

PARADIGM advances the patient engagement agenda. Integrated approach with initiatives like EUPATI and PFMD

## Why do we involve patients in HTA?

The HTAi values and standards looked at this issue from the HTA assessment point in time

<table>
<thead>
<tr>
<th>Relevance</th>
<th>Fairness</th>
<th>Equity</th>
<th>Legitimacy</th>
<th>Capacity building</th>
</tr>
</thead>
<tbody>
<tr>
<td>Patients have knowledge, perspectives and experiences that are unique and contribute to essential evidence for HTA.</td>
<td>Patients have the same rights to contribute to the HTA process as other stakeholders and have access to processes that enable effective engagement.</td>
<td>Patient involvement in HTA contributes to equity by seeking to understand the diverse needs of patients with a particular health issue, balanced against the requirements of a health system that seeks to distribute resources fairly among all users.</td>
<td>Patient involvement facilitates those affected by the HTA recommendations/decision to participate in the HTA; contributing to the transparency, accountability and credibility of the decision-making process.</td>
<td>Patient involvement processes address barriers to involving patients in HTA and build capacity for patients and HTA organizations to work together.</td>
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[https://htai.org/interest-groups/pcig/values-and-standards/](https://htai.org/interest-groups/pcig/values-and-standards/)
What about during Early Dialogues?

Early dialogues...

- ... are years before a HTA decision-making point
- ... are about complex decisions on study designs
- ... when there is little available evidence
- ... and are confidential discussions

Is patient involvement at this point in time appropriate?

How can it be implemented?

How can it add value?

Does it really make a difference?

Question for the audience...

Nicholas Brooke
Scientific Advice at NICE; rationale for patient involvement

Heidi Livingstone, Senior Public Involvement Adviser

Why do companies seek NICE Scientific Advice?

A: to increase: the likelihood that the company’s clinical development studies and other plans meet NICE evidence requirements

How does it help if patients participate in NICE Scientific Advice projects?

A: because it increases: the likelihood that the company’s clinical development studies and other plans meet the needs of patients
Why do we involve patients in Scientific Advice?

Patients

• The treatment is ultimately for them
• Can influence how clinical trials are set up
  • To provide the best evidence that the proposed outcomes meet patients’ needs

Companies

• Get powerful feedback on their decisions early in a product’s development

Stages of patient involvement

1. Briefing book
   Include patients?

2. Profile of patient/s wanted
   Patient identification

3. Face to face meeting

4. Brief chair

5. Telephone interview

6. Input into draft report

7. Final report sent to company

The patients can decide whether they want to participate in all 3 stages (preferred).
Scientific Advice Face to Face Meetings

Typical Issues Raised for NICE Scientific Advice where patients can help.

Value Proposition
Clinical Trial Programme

- Study population and subgroups
- Position of new treatment in the treatment pathway
- Comparators, i.e. current treatments available in the NHS
- Acceptability of proposed outcomes
- Measures of Quality of Life (and when to measure)
How patients can help with quality of life – when and what to measure

Varies from condition to condition:

• cannot complete EQ5D data during an attach or episode and may have to capture it retrospectively
• suggested measuring quality of life weekly, for other conditions monthly was considered manageable
• suggested it would be needed only once and at what point that should be

Additional types of measures suggested:

• tiredness additional questionnaire (interrupted sleep, insomnia, fatigue)
• cognitive function

Other examples of what patients can bring

• Why a tablet might not be an option for patients with certain conditions
• Why the six minute walk test doesn’t mean much to patients
• Why some population groups, or individuals, might not want a particular treatment
Feedback

- Company: Patient contribution unique and valuable
- Patients: Positive experience
- Other stakeholders: Value patients’ insight
- NICE Technical team: Patients can provide insight and additional information nobody realised was missing

Thank you!
Principles of patient involvement in EUnetHTA EDs

- **Patient perspective essential for EUnetHTA EDs:**
  - At the time forming the advice
  - Respect Conflict of Interest and Confidentiality rules

- **Providing unique insight of living with the condition to ensure recommendations meet patient’s need**
  - Advising on the signs and symptoms that have the greatest impact on their functional and psychological aspects of living
  - Impact on quality of life for patients and carers
  - (available) treatment & treatment expectation

- **Acceptability to participate in the proposed trial**
## Testing 3 possible approaches

<table>
<thead>
<tr>
<th>Approach</th>
<th>Patient contribution deliverables</th>
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</table>
| **Approach 1:** Individual patient/patient’s representative - interviewed regarding the disease and their experience | - Minutes of the interview  
- Mention of patient contribution in final EUnetHTA recommendations  
- Feedback questionnaire |
| **Approach 2:** Approach 1 + discussion with local HTAB regarding submission file (without applicant) | - Minutes of the interview  
- Mention of patient contribution in final EUnetHTA recommendations  
- Feedback questionnaire |
| **Approach 3:** Patient expert; Approach 1 + discussion with all participating HTABs regarding the submission file and participation in the F2F meeting with the Applicant | - Minutes of the interview  
- Review final EUnetHTA recommendations  
- Feedback questionnaire |

## EUnetHTA experience thus far…

10 of 14 completed EUnetHTA EDs with patient contribution following the 3 approaches:

1. 6 **interviews with patients** (France, UK, Spain)

2. 8 **interviews with a national patient representative**  
   (German patients’ representative involved in any ED in which G-BA participates)

3. 4 **EU patient representatives participating to overall ED process**
Method

An analysis based on feedback collected from 7 patients:

1. 5 patients (3 French, 1 Spanish and 1 English) (approach 1)
2. 1 German representative patient (approach 2)
3. 1 EU representative (approach 3)

**Approach 1:** Individual patient/patient's representative - interviewed regarding the disease and their experience

**Approach 2:** Approach 1 + discussion with local HTAB regarding submission file (without Applicant)

**Approach 3:** Patient expert; Approach 1 + discussion with all participating HTABs regarding the submission file and participation in the F2F meeting with the Applicant

Preparation for the ED

<table>
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| **While 5/7 patients never received training**  
- Quite clear information in ED general objectives  
  • 4/7 very satisfied  
  • 2/7 mostly not; 1/7 not informed at all  
- Quite clear understanding of what is expected from them  
  • 5/7 yes completely  
  • 1/7 mostly not | • Training: using different tools (EUPATI, national training tool...) and supports  
• A list of definitions at the beginning of the questionnaire |

As a reminder, all patients have been contacted by a patients’ organisation
Interviews

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| - Positive feedback on the phone interview, and their overall interaction with EUnetHTA  
- Large use of the questionnaire to prepare the interview (5/7 used it)  
- Appreciate open questions with opportunity to develop topics at their convenience  
- Patient had enough opportunities to express their opinion  
- Quite confident of the impact of their contribution  
Further access to Briefing Book and final recommendations requested | Translation of the questionnaire in native language for HTAi questionnaire and feedback questionnaire  
|  
* Briefing Book at disposal of interested individual patient?  
* List of Issue and Final recommendations to be shared Systematically with patients representatives |

Face-to-face meeting

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</table>
| • Interest in participating in F2F (because of the psychological impact of their physical presence)  
• Appreciate the opportunities for reactive statement | • Participation to F2F meeting proposed to individual/national representative with simultaneous translation .... |
## Time investment and administrative tasks

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<tr>
<td>Clear understanding of the confidentiality agreement</td>
<td></td>
</tr>
<tr>
<td>No difficulties to complete the DOICU and contract documents</td>
<td></td>
</tr>
<tr>
<td>Investment: minimum of half day to review the Briefing Book and only few hours to prepare the interview</td>
<td>Clarify payment and exchanges via IT system</td>
</tr>
<tr>
<td>No major burden of administrative task but still possibilities for improvement</td>
<td></td>
</tr>
</tbody>
</table>

Thank you!

EUnetHTA ED Secretariat: eunethta-has@has-sante.fr
## Patient involvement in Early Dialogues

Neil Bertelsen,
Chair HTAi Patient & Citizen Involvement in HTA Interest Group

### PARADIGM workshop on ED

- On 19th October 2018, eleven representatives from HTA bodies came together to discuss patient involvement in Early Dialogues, the current challenges in implementing patient involvement and the potential solutions (agencies from UK, Canada, Norway, Sweden, Spain, Italy, France and EUnetHTA)

| The rationale for involving patients and/or advocates in the early dialogue process | The current challenges of initiating a patient involvement process in ED | The current experience of involving patients and the challenges that have been identified so far | The resources or tools that would be useful in solving the identified challenges |
Agencies attending were very supportive of patient involvement in ED

Patients can make more impact in ED than in later assessment processes
Patients can help us capture the right thing in the right way at the right time
Prevents companies to make expensive mistakes
Ensure patient oriented research

Impact / Right timing

Improves advice given

Hear directly from patients about what matters to them
Unique insights from patients
Helps other stakeholders understand patient expectations
Provides perspective of the end-user
Patients know the difference between the “textbook” and reality

Rationale for patient involvement in ED

Directly relates to patient health
Treatments are for patients, so they need a say

A directly affected stakeholder

Transparency

Good governance
Transparent process

But they hear a lot of objections and there are implementation challenges

Objections often heard

Will bring subjective point of view
Not scientific enough
Will not know what is scientifically relevant

Not objective enough?

Patients will not understand early dialogue objectives and so cannot participate properly
Discussions will be too complex

Too complex for patients

Conflicts of interest and confidentiality

Need to keep discussions and information confidential

Challenges in implementation

Difficult to find the ‘right’ patient
‘Big’ diseases could have louder voices than ‘small’ ones

Resources needed

Who is the patient representing?
Difficult to identify representatives that match profile needed

Need to know ‘how’

Needs too much support time
Need the right skills / staff / training
Takes a lot of time to do this right

Lack of experience in incorporating patient perspectives
So to make patient involvement in Early Dialogues a reality we need...

To clearly articulate the rationale for patient involvement

Build processes that overcome the implementation challenges

Create consistency and predictability across patient involvement in ED processes

Have a menu of methodologies and approaches that could be applied

Set a baseline standard of patient involvement in Early Dialogues

Areas of immediate need

Standard, generic tools that can be shared across HTA bodies...

- Patient finding and recruitment
  - Patient finding case studies
  - Patient capability criteria guidance
  - Patient consent process
  - Patient guidance and ethical guidance

- Guidance and standards on patient interviews
  - Standard interview templates and guidance, adaptable for each ED
  - Guidance on skills needed for the interviewer

- Minimum standards framework
  - Framework of methods with guidance
  - Guidance for chairs
  - Guidance for patients
  - How to meet the needs of vulnerable groups

If any of you have examples of tools and resources in these areas (not necessarily for HTA or ED), then please share them if possible (nb@neilbertelsen.com)
Thank you
Neil Bertelsen

Question for the audience...
Nicholas Brooke