Can and should evidence / preferences collected from patients tilt the board in favour of accepting a new technology?

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Panellists: Paola Kruger, Giovanni Mauri, Andrea Marcellusi

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PATIENT FOCUSED MEDICINES DEVELOPMENT

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Welcome and introductions

MODERATOR	**Elisabeth Oehrlein, PhD, MS** Senior Director, Research and Programs, National Health Council, Washington, DC, USA
Patients	**Paola Kruger, MA** Patient Expert-EUPATI Fellow, Accademia dei Pazienti/EUPATI Italia, Rome, Italy
HCPs	**Giovanni Mauri , MD** Interventional Radiologist, European Institute of Oncology, Milan, Italy
HTAs	**Andrea Marcellusi, PhD** Faculty of Economics, Centre for Economic and International Studies (CEIS)-Economic Evaluation and HTA (EEHTA), University of Rome Tor Vergata, Rome, Italy

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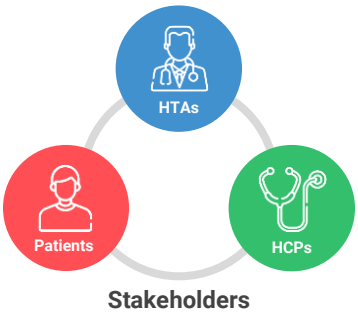
Today's focus

Explore challenges and opportunities in incorporating patient insights into HTA deliberations from a multi-stakeholder perspective



Discuss the 'weight' of the patient voice in decision making using a real-life case study to facilitate relevant and timely debate

Housekeeping

PLEASE SILENT OR SWITCH OFF CELL PHONES

PLEASE FEEL FREE TO ASK QUESTIONS!

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Case study:

Ultrasound-guided ablation techniques in nodular thyroid disease

Giovanni Mauri

Patient history (1)

- 57 year-old female
- No history of other diseases
- In 2005: diagnosis of thyroid nodular goiter, with large right lobe thyroid nodule
- Fine needle aspiration: benign nodule (thy 2)
- No symptoms

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Patient history (2)

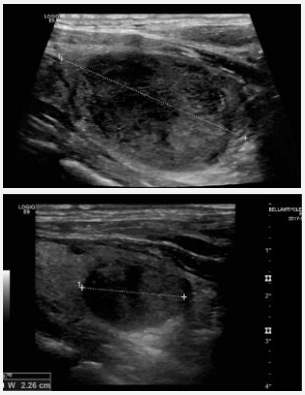
- Slow growth over time of the right thyroid nodule
- In 2012: started complaining of neck discomfort (dysphagia) and aesthetic concerns
- Normal thyroid function tests
- Endocrinologist and surgeon suggested surgical thyroidectomy
- The patient refused, with the desire of avoiding invasive surgery and maintaining thyroid function

Ultrasound-guided radiofrequency ablation

- In 2013: ultrasound guided radiofrequency ablation
- Procedure is performed under local anesthesia, in outpatient setting
- A small needle is inserted into the nodule, and radiofrequency energy is applied to achieve the death of nodule cells

Ultrasound-guided radiofrequency ablation

- After 2 weeks: initial reduction of the nodule volume and improvement in symptoms
- At 6 months from treatment: complete disappearance of symptoms. Normal thyroid function. Volume reduction from 18.9 ml to 3.06 ml
- At 5 years from treatment: no symptoms, normal thyroid function



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Key messages: ultrasound-guided ablation techniques in nodular thyroid disease

- Ultrasound-guided ablation techniques are new minimally-invasive techniques that can be used to treat benign thyroid nodules
- Costs are similar to surgical thyroidectomy, however,QoL for the patient is improved
- Since costs are the same there is no incentive for hospitals to introduce it and change supplies and train staff
- However, patients prefer it on the basis of better QoL

Panel and Participant Discussion

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Multi-stakeholder perspectives

VALUE

EVIDENCE

What is the value of incorporating patient evidence and preference into HTAs?

Do we have metrics and/or evidence?

Is there economic value?

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Patient and HCP perspectives

What are the potential consequences of NOT incorporating patient evidence/preference?

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HTA perspective

METHODOLOGY

ALIGNMENT

What do HTA bodies consider robust patient evidence? What methodology should be used?

Does patient evidence/preference carry equal weight as other 'traditional' evidence – if not – why not?

Are different HTA bodies aligned in incorporation/use of patient evidence?

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Multi-stakeholder perspectives

Is better alignment and/or standardization required?

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Multi-stakeholder perspectives

What tools already exist?

GUIDANCE

TOOLS

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What tools already exist ? The Toolbox

Inserting the Patient Voice	Patient Perspectives on Disease Impact and Treatment Options: A Stratification Tool (NHC) Patient-Journey Mapping Toolbox (NHC) PFDD Meeting Resources (FasterCures) Real-World Evidence (NHC)
Capacity Building	European Patients' Academy Toolbox (EUPATI) Clinical Outcome Assessment (NHC)
Guidances/Roadmaps/Rubrics	FDA PFDD Guidances The Patient Engagement Quality Guidance (PFMD) Tackling Representativeness: A Roadmap and Rubric (NHC)
Planning	Responsible Research and Innovation Toolkit (EPWS)
FMV and Legal	Fair-Market Value Calculator (NHC) Sponsor-Patient Interactions During Drug Development: Good Practice Insights on Patient Engagement (NHC) Reasonable Legal Agreements (PFMD)

NATIONAL HEALTH COUNCIL

PFMD Patient Engagement Quality Guidance

76 participants from **51** organisations participated in **9** multistakeholder meetings (2016–2018).

A shortlist of **20** relevant PE initiatives (from **170** screened) were identified

The UK National Standards provided the main framework for the first draft of the PE Quality Guidance.

7 key PE Quality Criteria were identified

Public consultation yielded **67** responses from diverse backgrounds.

The PE Quality Guidance was agreed to be useful for achieving quality patient engagement in practice. It is comprehensive, understandable and easy to use.

8 initiatives were selected from various sources to be included in the BOGP based on their demonstration of one or more PE Quality Criteria.

PE Quality Guidance pilots are underway

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Multi-stakeholder perspectives

GUIDANCE

TOOLS

What tools or guidance are needed?
Who should be involved in co-developing these tools?

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Discussion

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Thank you!

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