


European Federation of  
Pharmaceutical Industries  
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## The role of HTA in evaluating future medicines: EFPIA principles

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## HTA: back to the roots

“**Health technology assessment (HTA)** is a multidisciplinary process that summarises information about the **medical, social, economic and ethical** issues related to the use of a health technology in a systematic, transparent, unbiased, robust manner.

Its aim is to inform the formulation of **safe, effective, health policies** that are **patient focused** and seek to achieve **best value**”

EUnetHTA, European Network for HTA ([www.eunethta.net](http://www.eunethta.net)) 2007

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### What Do Different Stakeholders Value in a Health Technology? Results of a Small Group Analysis

*Devidas Menon (Canada), Marjukka Makela (Finland), and members of the HTAI Policy Forum Value*

**Background:**

In HTA, the “value” of a health technology is most often assessed by means of an economic evaluation” as through determining financial implications”. At the same time it is becoming recognized that the perceived value of a new technology will depend on whether one is a doctor, a patient, a family caregiver, or a health professional providing care to a patient, i.e. it is perspective-dependent. Therefore, there has been considerable debate recently on the relative value attributable to different stakeholders, particularly as it might influence price-setting and decisions made by governments on new health technologies, and therefore, access to them. It has been a topic of specific interest to EFPIA Policy Forum whose members include government decision-makers, payers

**Results:**

The table shows the results of this exercise.

- Lack of alternatives” was seen to be a very important value of new technologies by all stakeholder groups. All groups also rated “safety” and “effectiveness” similarly.
- The groups differed in their view of financial aspects (cost of intervention) and “budget impact”, with patients, professionals and employers considering these to be not important aspects of value. Clearly, managers and payers thought differently about these aspects.
- Statistical analysis of the data, using the kappa coefficient, showed that there was “very good” overall agreement between payers and government (0.84), good agreement between managers and payers (0.62) and moderate agreement between managers and government (0.51). This seems to be driven by the relative priority placed by cost and expenditure considerations. There was at best fair agreement between any other pair of groups.
- Patients and professionals agreed substantially on their judgments of value: the exceptions were “convenience of use” which mainly was thought to be important to patients, professionals and industry, but seems not to be so to professionals, and “feasibility” (the feasibility of providing the technology in existing surroundings, i.e. training, space, etc. needs), which was important to professionals but not to patients.

ATTRIBUTE	Pa	Ma	Pr	Em	Gov	Ind	Doc	Pa	Stk
Lack of alternatives	3	2	3	3	3	3	3	3	3
Safety	3	2	3	2	2	2	2	2	2
Effectiveness	3	2	2	2	2	2	2	2	2
Convenience of use	2	2	1	1	1	1	1	1	1
Feasibility	3	3	3	2	2	2	1	1	2
Impact	2	2	2	1	1	2	2	2	2
Cost	1	1	1	1	1	1	1	1	1
Budget impact	1	1	1	1	1	1	1	1	1
Health	1	2	2	1	1	1	2	2	2
Effectiveness	1	2	2	1	1	1	2	2	2
Health Impact	1	2	1	1	1	1	1	1	1
Management	1	1	2	1	1	1	1	1	1
Government	1	1	1	2	2	1	1	1	1



**Conclusions:**

“Value” is clearly in the eyes of the stakeholder. Stakeholder discussions that are made from the perspective of payers or governments, should duly regard attributes of technologies that patients or professionals consider to be of value.

**References:**

(1)Pharson SD and Brouha MD (2005). Quality, innovation, and value for money. *AMA* 294:2610-2627.

## Assessing « Value » in Healthcare: EU terms of reference or....an attempt

### EU Commission High Level Pharmaceutical Forum agreed definitions:

- **Efficacy: “Can it work?”**
- **Effectiveness: “Does it work in practice?”**
- **Relative Effectiveness: “Is it worth it?”**

### The unanswered questions:

- **“Is the introduction of this drug worth its costs?”**
- **“For whom is the value?”**
- **“Can we afford it?”**

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## Industry perspectives



- Recognise the **willingness** of Governments to assess the clinical and/or cost-effectiveness of medicines
- Support a **patient-centered** healthcare system (timely and equal access to care)
- Call for processes and principles that reward **value** and **innovation**
- Position HTA as a springboard to generate **better health outcomes**
- Point out that evaluation mechanisms **often not in line** with these goals

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## Key principles for discussion



- **HTAs should be based on a clear view of what constitutes value**
  - clarity and consensus on the criteria against which therapeutic progress (or value) can be identified
  - progress could be innovation that is of value to sub-groups of patients
- **HTAs should be transparent and balanced**
  - evaluating body independent of the payer
  - clarity and consistency on methodology, criteria used and data required
  - efficient and independent handling of appeals
- **HTAs should be based on early and inclusive dialogue, including with patients**
  - what do authorities expect in terms of therapeutic added benefit?
  - better dialogue between industry and authorities
  - inclusive - medical sector, patients, industry

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## Key principles II



- **Evaluations should allow new data to be considered**
  - 'one size fits all' approach to the timing of appraisals too crude
  - data needed is often real-life clinical use data
  - submit health outcomes information throughout a product's lifecycle
  - systems should enable real-life benefits of medicines to be evaluated
- **Flexibility is required in handling uncertainty**
  - flexible partnership approach between payers and industry
  - enjoy early, reimbursed launch of products
  - further clinical outcomes data may affect reimbursement level
  - proper implementation of partnership approaches key
- **Comprehensive understanding of the benefits of a drug in disease management is needed**
  - perspectives on a drug's value should be broad
  - evaluations should take into account indirect benefits

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## Key principles III



- **Payers should commit to rewarding added value**
  - reward for innovation can come in different forms – price-setting or readjustment, volumes, therapeutic guidelines, speed of access
- **HTA outcomes should be implemented**
  - where evaluation is positive, payers should commit funding to encourage implementation
  - HTA guidance should enable physicians sufficient freedom to address individual clinical situations
  - good communication to encourage implementation

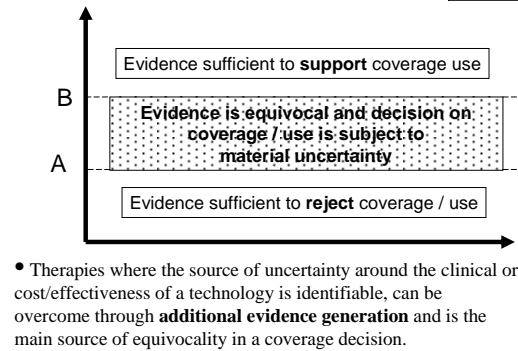
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## Assessing the « Value » of innovative drugs: where Uncertainties lie



- Therapies which have the potential to offer **significant health or economic benefits**;
- Therapies where the **balance of uncertainty** suggests that the outcomes of the CED process will confirm the presence of a significant clinical or economic benefit;



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## Managing « Uncertainty » from a Public Health perspective



- What is “**sufficient**” at different assessment points?
- How can the **timing of data collection** and associated **regulatory and payers decisions** be improved to capture the benefits, risks and value to optimize patient care?
- How can **pre- and post-marketing evidence** requirements be better adapted to serve the need of physicians, patients and payers?
- How can we **ensure a regulatory and reimbursement decision making process** that better rewards and promotes innovation/progress in medicine that benefits patients?

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## “The first step to wisdom is getting things by their right name” Chinese proverb



- **Policy-makers face tremendous challenges when making recommendations for the adoption of new therapies.**
- **The perceived value of a therapy** may differ depending on whether one is a payer, a patient, a family member, or a physician **involved in the healthcare of a patient.**
- **Where healthcare allocation decisions are judgemental and values driven, the fullest possible transparency and stakeholder involvement are basic and necessary components.**

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