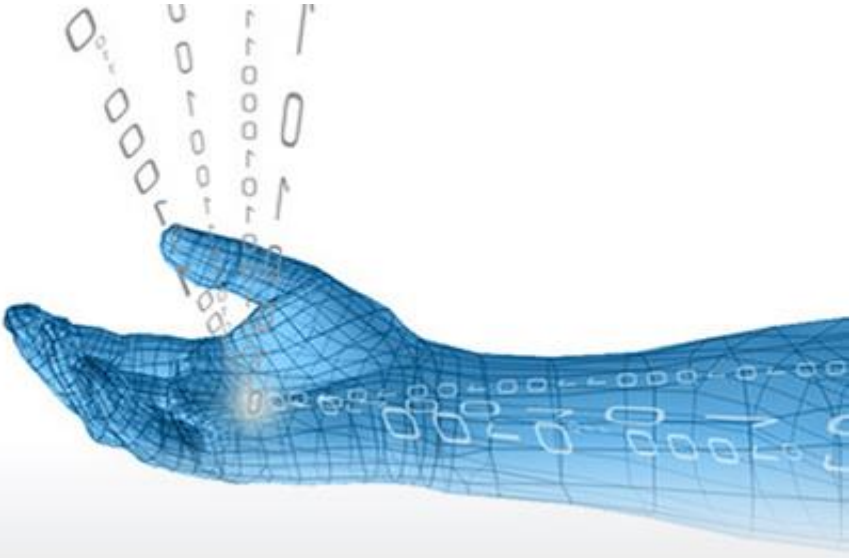
Advanced Innovation
Health Technology
Assessment



Building a Framework for a Societal Benefits Approach to Health Technology Assessment

Workshop held at the
Marivaux Hotel and Congress Centre, Brussels, Belgium
on 4 February 2013

EXECUTIVE SUMMARY

epposi Partnering for Healthcare Policy

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AIP-HTA PROGRAMME PARTICIPANTS

Observer members
European Commission – DG Health and Consumers (SANCO)
European Parliament

Patients' Organisations
EATG (European AIDS Treatment Group) • ECPC (European Cancer Patient Coalition) • EFCCA (European Federation of Crohn's and Colitis Associations) • EFNA (European Federation of Neurological Associations) • EGAN (European Genetic Alliances' Network) • EURORDIS (Rare Diseases Europe) • European Women's Health Institute • GAMIAN-Europe (Global Alliance of Mental Illness Advocacy Networks - Europe) • IBTA (International Brain Tumour Alliance) • IPOPI - International Patient Organisation for Primary Immunodeficiencies • Retina Europe • WFIP (World Federation of Incontinent Patients)

Science, Academia & Public Administrations
Council for HC and Consumption • Delft University of Technology • ESHG (European Society for Human Genetics) • Fit for Work Europe/ The Work Foundation • HTAi • NICE (National Institute for Health and Clinical Excellence) • Office of Health Economics • RAPS - Regulatory Affairs Professionals Society • University of Manchester • University of Southern Denmark • Institute of Medical Technology Assessment, Erasmus University Rotterdam

Industry and Payer Community
Abbott • AIM (Association Internationale de la Mutualité) • Amgen • Baxter • CSL Behring • EDMA (European Diagnostic Manufacturers Association) • Eli Lilly • F. Hoffman La Roche • Gilead • GSK • Johnson & Johnson • MSD • Pfizer • Shire

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Key words

societal benefits approach - societal value - Patient-Defined outcomes - continued economic activity - significant others - cross-sectoral policy making - all stakeholders involved - psychological aspects - ethical aspects - silo budgeting - budget rationalisation

Knowledge Partner

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EXECUTIVE SUMMARY

Introduction

Over the course of the past two years, Epposi has been working on developing and building with its members a multi-stakeholder consensus for a framework for a societal benefits approach to HTA, the core output of its **Advanced Innovation Programme in Health Technology Assessment (AIP-HTA)**. Programme members are equally weighted between patients' organisations, science and industry, and include observers from the EU institutions, HTA agencies, payers and ethicists.

The AIP-HTA is one of four Epposi work programmes – alongside Chronic Conditions Management, Innovation in Healthcare and Rare Disease - which are designed to be citizen-centric and make a positive contribution to European health policy-making, by providing members and the wider public with high-quality independent research, capacity-building, knowledge exchange and dissemination with the aim of bridging the gap between innovation and improved public health outcomes.

Epposi focuses on a multi-stakeholder approach to ensure that all relevant elements of HTA processes and content, which can have a direct societal impact, are common to most agencies in Europe, as well as being enshrined in EUNetHTA's HTA Core Model®, and sufficiently well expressed and measurable to be integrated in HTA frameworks at national level across Europe.

The main purpose of the question currently addressed by the Programme has been to complete the identified gaps in the social elements of EUNetHTA's HTA Core Model®, which are significantly less developed than the more quantifiably measureable elements.

Workshop objectives

The workshop was convened to enable all stakeholders, through breakout sessions, plenary discussion, panel debates and consensus-building exercises, to:

- Refine and validate the proposed domains and sub-domains of Epposi's societal benefits approach to HTA
- Agree the final outline of the framework
- Outline the key steps towards a roadmap for potential implementation of the framework
- Identify the added value and benefits of the framework
- Confirm the next steps.

Key outcomes

Three key outcomes were achieved at the workshop:

1. The proposed 10 domains of Epposi's framework for a societal benefits approach to HTA were examined by all stakeholders and revised to 9, and re-clustered to focus on HTA content and process.
2. It was agreed that the framework met the criteria of clarifying the societal elements in EUNetHTA's HTA Core Model®, which Epposi had found vague, and all stakeholders would now work with Epposi to find a workable solution.
3. The solution (or evidence of the solution existing in any other countries) will be consolidated in a White Paper to be used as part of the public affairs outreach programme.

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The revised 9 domains and clusters

It was agreed to add a new, separate domain – significant others[1] - and that the domain of 'value' should encompass all the others.

Value		
HTA content **Ethical Aspects** **Psychological Aspects** **Patient-Defined Outcomes** **Continued Economic Activity**	*HTA process* **Governance** **Cross-Sectoral Policy-Making** **All Stakeholder Involvement**	**Significant Others**

Keynote perspectives on societal value in Europe

Experts in health economics, conditional reimbursement and assessment and appraisal frameworks for rare disease were invited to give the latest updates in their fields.

***Health economics*: Prof Hans Severens**, Professor of Evaluation in Healthcare at the Institute of Medical Technology Assessment and Institute of Health Policy Management at Erasmus University in Rotterdam, a member of the Dutch national health council and a former Board Director of ISPOR[2], highlighted three current approaches and challenges to getting societal values into the cost-effectiveness equation:

- **Significant others:** society needs to look not just at the effect, cost and benefits of a technology on the patient but also the 'spin-off' on family members and caregivers – the family effect. The life years gained can then be calculated not just in the patient but in the other family members which can be put into a cost per QALY or cost per life year calculation. The Institute at Erasmus is currently developing an instrument – Carer Qol - which describes what happens to a carer as a result of the illness of the person they are taking care of. How to put in the equation the costs and the health-related quality of life? So far, the conclusion is that wellbeing is a separate item – it cannot be added to the health effects in patients.
- **EQ5D[3] measurement:** derived from a patient questionnaire about their perceptions of their health on the day they complete the form. Patients choose between three options which rate their ability (no problem to…, some problem in …, unable to…) under categories such as Mobility, Self-Care, Pain/Discomfort and Anxiety/Depression. This results in a simple 1-3 coded scoring system to create a value set (work is ongoing to expand this to five options per category). A tariff is then used to calculate a utility value.
- **Personalised/stratified medicine: i**t is well known, for instance, from trial data and sub-group population analyses that some people respond to a treatment and some do not. More startling is that 90% of drugs do not work in 40% of patients. Health economists are therefore looking at ways to deal with this variability within a patient population, and other factors such as how to cope with health losses (some patients responding negatively to a health technology), to take a more personalised medicine evaluation into account[4] to calculate an optimal cost-effectiveness

[1] The effect, cost and benefits of a technology not just on the patient but also the 'spin-off' on family members and caregivers.
[2] International Society for Pharmacoeconomics and Outcomes Research: www.ispor.org
[3] EQ-5D™ is a standardised instrument for use as a measure of health outcome. www.euroqol.org/
[4] Grutters et al. Acknowledging patient heterogeneity in economic evaluation: a systematic literature review. PharmacoEconomics 2013; 31: 111–123.

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and optimal implementation of the technology. Ultimately, the final decisions taken are as much political as economic. But, he concluded by saying that "we can still put societal values *explicitly* into the assessment phase."

***Patient policy-making*: Understanding patient need** was at the core of the **AGNSS project (Advisory Group for National Specialised Services - England),** specifically in relation to rare diseases. However, as **Josie Godfrey**, Head of Policy and Coordination for AGNSS's National Specialised Commissioning Team, pointed out, the process of developing a framework that has consensus, identifying a range of criteria that are relevant to the appraisal and providing the evidence and methods of assessment of those criteria, is something that is transferable the mainstream population - but the specifics of those criteria have to be determined by the particular issues and context.

AGNSS formulated four questions about the product, service or technology to see how it addressed patient need – which could be equally applicable to the non-rare disease patient:

1. **Does it work**? (What is the severity/burden of the disease? What is the capacity of the patient population to benefit? Is there evidence of clinical efficacy?)
2. **Does it add value to society?** (The most difficult question for the committee was to determine what should be included. There were some specific questions to do with innovation and the extent to which it contributed - difficult to define, but included to allow a wide range of perceptions and not to prejudge responses.)
3. **Is it affordable to society?** (This aimed to look at the overall cost, not just the cost per patient.)
4. **Is this the best model of care?** (Is this the best way of delivering the service? Does the intervention fit? What is the feasibility of introducing a product into the health system?)

Conditional reimbursement I*: Dr Maartje Niezen**, Research Fellow, School of Social and Behavioural Sciences, Tilburg University (Netherlands), reported on her research into two different forms of conditional reimbursement – ***conditional financing and ***restriction of indications***. Through data collection conditional reimbursement or access allows for the further research of medicines that seem promising but whose effectiveness has not yet been fully established.

Criteria for conditional reimbursement: research goals should be clear and formulation of a clear research design; the context of the healthcare system should be taken into account; for a societal benefits perspective, patients and other stakeholders must be involved and the burden of disease taken into account; and data are collected for registries.

The drawbacks: a lot of money is potentially given to researching medicines which turn out to be ineffective. Once medicines are reimbursed, even conditionally, it is very difficult to get rid of them from the approved list or package - one of the key problems (in her view) of the conditional access system adopted in the Netherlands from January 2012.

***Conditional Reimbursement II*: Pascale Brasseur**, Chair, Medtronic HTA working group and Reimbursement Director, Medtronic Europe, addressed the common view that the **reimbursement of medical devices** is more difficult than that for pharmaceuticals. This is not necessarily the case. It is possible to conduct clinical trials and even double-blind trials for medical devices to obtain evidence for reimbursement, she noted and highlighted other challenges:

- The capacity of the whole HCP team to affect the outcome. This could mean how and which pre-op imaging or scanning is chosen, anaesthesiology, post-care nursing and the whole follow-up protocol.
- Looking at whether high-volume centres have better outcomes than low-volume.

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- A need for new forms of studies - ***pragmatic trials*** – when there is no alternative as the sample size is too small for a conventional trials and start building evidence bit by bit.
- The requirement for ***post-reimbursement studies*** (eg France) where manufacturers are asked to come back with the results of new technology (implants, surgery etc) after two or three years in order to assess the efficacy of the device. Reimbursement may not be guaranteed longer-term and might be revisited annually in some cases.

Validation and consensus-building exercise

A voting exercise was carried out to gauge participants' reactions to the morning's presentations and to give their own stakeholder perspectives on a societal benefits approach to HTA.

All stakeholders who are involved in the HTA process (industry, patients' organisations, NGOs, HTA agencies, science, payers) should collaborate actively and each stakeholder's views on final recommendations should be of the same value regardless of their different interests and roles.

Strongly agree	14%	
Agree	**31%**	**no majority view**
Neither agree nor disagree	17%	
Disagree	29%	
Strongly disagree	9%	

Patient involvement and empowerment are essential for all HTA decisions, since patients can provide insight not only into the impact of diseases but also into the benefits of the treatments assessed.

Strongly agree	**59%**	**}**	
Agree	**26%**	**}**	**85% in favour**
Neither agree nor disagree	15%		
Disagree	0%		
Strongly disagree	0%		

Life-saving treatments should have different thresholds than treatments with moderate health outcomes. An approach which looks beyond QALY criteria is preferred.

Strongly agree	**31%**	**}**	
Agree	**40%**	**}**	**71% in favour**
Neither agree nor disagree	17%		
Disagree	6%		
Strongly disagree	6%		

Workability, including absenteeism and presenteeism, has a direct economic impact and should be part of an HTA assessment. Voluntary or charity work should also be part of an HTA assessment, since it has indirect impact on costs and direct impact on societal benefits to patients.

Strongly agree	**29%**	**}**	
Agree	**49%**	**}**	**78% in favour**
Neither agree nor disagree	14%		
Disagree	0%		
Strongly disagree	9%		

In your opinion, which aspect of social value appeals most to you?

Public preferences (society as a whole) are more important than individual preferences	3%
Values of equity, efficiency, compassion and quality of life	17%
Healthcare should be distributed according to need and not according to the ability to pay	20%
Everyone counts. We use our resources for the benefit of the whole community and make sure nobody is excluded or left behind	14%

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Assessment of impacts of a health product should go beyond clinical and economic ones	14%
Maximise health of the population, subject to limited resources	**31%**

Next steps

Jacqueline Bowman-Busato, Executive Director of Epposi, acknowledged the divergence of views among the stakeholders and the ongoing work needed to refine and clarify the domains of Epposi's societal benefits approach.

In particular, she highlighted five areas that will be addressed:

1. Clustering the domains into process and content.
2. Separating assessment (evidence) and appraisal (setting criteria for decision-making). Assessment must stem from the appraisal. In Epposi's originally proposed domains there was an overlap between HTA Methodologies and Assessment. Epposi will now focus on HTA content and process.
3. Looking at additional domains to be included. First suggestion arising from the workshop: Significant Others.
4. Moving forwards with the clusters will facilitate looking at interlinkages. Very low interlinkages between assessment and final decision-making – good evidence does not mean a positive political decision.
5. Economic effects need to be measured before appraisal/decision-making can be included – cross-sectoral policy-making is critical so that benefits to one part of society are not financed at a loss to another (eg health budget bears brunt of employment issues).

Key 2013 planned outputs

A White Paper outlining the 9 domains of Epposi's societal benefits approach to HTA (taking into account the outcomes of today's workshop) will be published encompassing all the research and stakeholder consensus to date followed by a dissemination campaign and follow-up policy briefings in the domain of value.

Epposi will continue its collaboration with other HTA specialist groups (HTAi, ISPOR and EUNetHTA) and will establish the ISPOR Special Interest Group on Rare Diseases and HTA. Epposi will also be submitting a response to EUNetHTA public consultation of the Policy for the HTA Core Model.®

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