



Health Technology Assessments in Ireland

– where are we now?

IPPOSI
Irish Platform for Patients' Organisations,
Science and Industry

Index

1. Conference Report	4
The Report and It's Aim	4
1. Executive Summary	5
Health Technology Assessment today, 2011	5
Key recommendations from the 2011 meeting	6
How to strengthen the patient voice in HTAs	7
2. Overview of the presentations	9
Stakeholders Standpoints – A common call from many perspectives	9
National Policy Perspective	10
Academic Perspective	12
Industry Perspective	13
A European Patient Perspective	14
3. Summary of discussions	17

Conference Report

The report and its aim

This report attempts to capture the rich discussion and debate which took place at an Irish Platform for Patients' Organisations, Science and Industry (IPPOSI) meeting on the 5th April 2011 entitled; 'Health Technology Assessments in Ireland – where are we now?'

It is clear from Ireland and abroad, from our Department of Health (DOH) and the Health Service Executive (HSE) to the European Commission (EC), that a new and robust culture of patient participation in decision making processes is to be expanded and promoted. Patients and patient groups are keen to join the HTA process debate!

This IPPOSI conference was designed to empower stakeholders to understand the HTA processes better and from each other's perspectives, to see areas of alignment, and to strengthen much needed efforts to make patients more active in HTAs.

Conference participants, including patient groups, policy makers and industry, outlined their strategies, areas of alignment, and policy concerns. As always, the IPPOSI conference put the patient at the centre. In reality everyone at the conference (like everyone in society) is or will be a patient. Conference recommendations to ensure we reach the shared goal of a robust, patient-centred, HTA process are outlined below.

1. Executive Summary

The conference on the 5th April 2011 was organised as a follow on from the November 2008 IPPOSI Conference which addressed three important issues:

- 1. Health Technology Assessments in Ireland,**
- 2. Ireland's Experience to date,**
- 3. Europe's Experience to date**

There was general consensus amongst participants at the conference that the HTA process in Ireland was about to change with the establishment of the Health Information and Quality Authority (HIQA) and the move towards HTA systems as the norm in the rest of the EU.

The 2008 conference explored future changing structures for HTAs in Ireland – examining the plans and how they will be developed in line with national and international practice. Some of the key points raised then include:

- Ireland needs to foster a consensus based approach to the development of HTAs rather than a prescriptive approach.
- The scope of HTA should not be limited to economic considerations only. Social, ethical, legal considerations are equally important.
- Patients and patient organisations need to be assisted and empowered to better understand HTA system and to formally engage in the HTA process.

Health Technology Assessment today, 2011

In Ireland today, the issues highlighted during the conference are quite similar to those of 2008. The scope of HTA's in Ireland is still quite limited as both the social and legal considerations of the individual patient in the HTA process are not widely considered. There has been limited involvement of patient organisations or patients in pharmaceutical HTAs while there has been some involvement in the overarching structure (HTA Advisory Board), more involvement is required. Hopefully, the announcement from HIQA that it will be developing a national HTA policy framework/legislation for the involvement of patients and patient organisations in the HTA process

will enable this to happen more systematically.

IPPOSI members are aware of the budgetary challenges and know that tough decisions are to be made in our healthcare system but broader stakeholder involvement in the HTA process will contribute to a more open and transparent process.

The HTAs are conducted by the same agencies as that described in 2008 report. HIQA conduct HTA's of national significance eg population based vaccination programmes, medical devices, screening. HTA's on new emerging pharmaceutical products are conducted by the National Centre for Pharmacoeconomics (NCPE) under HSE/IPHA agreement. Rapid HTA's are used as a tool by the NCPE to decide if a full HTA is required. Finally, there are guidelines in development with regard to 'Mini-HTA': to inform local level decision-making. This is a development which many members verbalised concern as equity of access in differing geographic areas could become an issue in the coming years.

Key recommendations from the 2011 meeting

- HIQA to drive national HTA Policy Framework/legislation for the involvement of patients and patient organisations in the HTA process
- Capacity and Infrastructure – there will be a considerable demand on HTA agencies (HIQA and NCPE) over the coming years and their capacity to deliver will be a challenge. Building HTA capacity should be prioritised to minimise access delays to treatment.
- Transparency and process clarity - while the process of how a HTA should be conducted is transparent, the decision to conduct a HTA and decision to grant access to treatment is not always clearly explained nor does it follow a systematic path ie. Decision to be taken in “Y” number of days and communicated by “X” to the public.
- Patient Involvement – patients and patients’ organisations need to be fully informed and able to participate in the HTA process.
- Development of training courses for HTA agencies and patient organisations (the why and the how of patient involvement). Concrete steps and resources to bridge the knowledge and language gap between HTA experts and patients is needed.



- HTA agencies need to develop tools to better value qualitative research which is most often at the basis of patients' evidence versus quantitative research.
- Concrete steps and resources to bridge the knowledge and language gap between HTA experts and patients are needed

How to strengthen the patient voice in HTAs

Focusing on the patient involvement, key points of the discussion focused on defining meaningful patient involvement as patients taking an active role in activities or decisions that will have consequences for the patient community, because of their specific knowledge and relevant experience as patients. The involvement must be planned, appropriately resourced, carried out, and evaluated, according to the values and purposes of:

- The participating patients or patient organisations
- Other participating organisations and funding bodies
- The quality of their experiences during the involvement activity

With 150 million EU patients with chronic conditions the urgency of effective patient involvement was emphasised and agreed. The main benefits of patient involvement are:

- Addressing the right issues/better knowledge
- Validating good practices
- Broader/different perspective particularly on quality of life issues
- Achieving objectives, that is better results, and the legitimisation of results
- Empowerment of patients
- Being a community/patient voice ensuring needs are understood

The main challenges of patient involvement are:

- Lack of legislative and/or policy framework
- Lack of/poor culture and tradition
- No methodology
- Attitudes and perceptions
- Resources and capability
- Communication



2. Overview of the presentations

On the 5th April 2011 key healthcare stakeholders came together to discuss the HTA system in Ireland and the role of the patient in HTA decision making. We worked to benchmark how HTAs, in principle and practice, have advanced as transparent and valuable aid to policy making in Ireland since our previous HTA conference in November 2008.

Dr. Ryan, HIQA, outlined developments in Irish health technology systems, starting from the establishment of HIQA, its role, current HTA structures, and her vision for HTA in Ireland.

Our speakers emphasised the importance and benefits flowing from the multidisciplinary nature of HTA. Professor Ciarán O’Neill, NUI Galway, began his presentation saying that “economists do not have all the answers”, highlighting that any new or existing health technology intervention is “part of a process, it connects to part of the health system and its effects ripple through the system”. Ciarán further added that “HTA is not just about ‘doing’, but the process itself continues to evolve”.

Ms Liuska Sanna, from the European Patients Forum (EPF) stressed the need for ‘meaningful’ patient involvement in the HTA process. The EPF have identified both benefits and challenges that need to be capitalised on in the HTA process. Patients bring a qualitative value rather than a quantitative value to decision making, but for patient involvement to be meaningful, Liuska emphasised that “measurability is important, and how this can be achieved is yet to be fully defined”.

Our IPPOSI conference addressed three important issues: (i) Health Technology Assessments in Ireland, (ii) Stakeholders Experience across Europe to Date, (iii) How to strengthen the patient voice in HTAs.

Stakeholders Standpoints – A common call from many perspectives

Patients, patient organisations, national policy makers, industry, clinicians and specialist academia were represented at the IPPOSI conference. While representation

was diverse, the issues of concern raised by each of the speakers strongly reflected a communality of themes. Focusing on improving patient outcomes, particular alignment occurred in the following areas:

- (i) The urgent need for greater transparency in the HTA process;
- (ii) Maximising our efforts towards optimally following NCPE / HIQA recommendations in both policy making and in patient participation;
- (iii) Optimally mitigating against the current threat to R&D as a result of certain deficits in the current HTA ecosystem;
- (iv) The urgent need for more robust patient capability development (training) in HTAs; and
- (v) Overcoming the current substantial deficits in the current ability of key stakeholder to efficiently access vital information.

National Policy Perspective

Dr Ryan outlined current HTA systems and structures in Ireland. In her presentation she told the story of the establishment of HIQA, its role, current HTA structures, and where she envisioned how HTA will progress in Ireland in the future.

Dr Ryan emphasised the novel, beneficial and complex cross functional nature of HTA processes in Ireland, e.g. medical, social, economic, ethical, medico-legal the social, and ethical elements. Dr Ryan reiterated the focus and objective of HTA as a discipline, “...to inform safe and effective health policies that are patient focussed and achieve best value”. Dr Ryan outlined a prime distinction between HIQA and its UK equivalent, to whom HIQA is frequently compared “the clear distinction from NICE is that HIQA provide advice and recommendations to the Minister (of Health), who ultimately makes the decision”. The concept of optimally rationing scarce resources among needy patients is difficult however the challenge is to find the balance between the clinical purist and the financial realist to achieve the fairest outcomes possible for the whole of society.

Dr Ryan shared insights on two relevant examples of the work of the HTA Directorate within HIQA, looking at the HPV vaccination programme, and secondly the recently published report on a population-based colorectal cancer screening programme.

Focusing on the colorectal cancer screening programme findings, one interesting note was that while the 'optimal strategy' was found to be highly cost-effective, the implementation of such a programme would involve a high resource constraint, and therefore the HTA team in HIQA were further tasked with designing a strategy to further leverage current resources to enable the roll out of the programme with minimal resource impact. Dr Ryan pointed out that this is a further area that differentiates the role of the HTA directorate of HIQA from its European counterparts, "most countries HTA structure stops at proving clinical & cost effectiveness, but for Ireland resource and allocation issues are also part of the continued process".

The cost of drugs to the state was the focus of several strategies for cost containment outlined by Dr Ryan in her presentation. Her central points included:

- The pharmaceutical industry is to be targeted through post-patent price reductions and reference pricing
- Community drug schemes (CDS) is to undergo a level of disinvestment
- Wholesalers are to see a reduction in margins
- Pharmacists are to undergo a change to their reimbursement model
- Patients are expected to incur a level of co-payments for their drug requirements
- Prescribers are to be induced toward generic prescribing and made more aware of opportunity cost issues.

To affect prescribing patterns, HIQA are currently working on a "National Clinical Guidelines" framework that will ensure that prescribers are following the most cost-effective treatment strategy, as recommended under these NCG's. NCG's will be supported by clinical & cost-effectiveness evidence as assessed through the HTA process.

In looking to the future, three areas of focus for the HTA directorate of HIQA centre on the national HTA policy framework, an increased focus on cost containment & disinvestment, and the development of clinical effectiveness guidelines.

Commenting on the political environment, Dr Ryan noted that "the proposed new 'Dutch Healthcare system' which forms the central tenet of our new government's healthcare policy, offers an opportunity to move away from the current situation

of very limited visibility or control over hospital budgeting practices, to an insurance system where there is greater scope for cost control. However, where HTA fits in that is currently unmapped”.

Academic Perspective

Echoing the importance and value of the multidisciplinary nature of HTA, Professor Ciarán O’Neill began his presentation by saying that “economists do not have all the answers”, and any new or existing health technology intervention is “part of a process, it connects to part of the health system and its effects ripple through the system” Ciarán further added that “HTA is not just about ‘doing’ per se, but the process itself continues to evolve”. To illustrate his point Ciarán exemplified the UK NICE initiative of ‘Reference Cases’, which he believes allow for substantially improved transparency and clarity in the HTA process.

Addressing the question of the role of the academic in HTA, Ciarán pointed to three core areas of demand

- application of HTA techniques
- development of techniques, and
- development of capacity (course through-put of persons qualified to work in HTA)

Ciarán commented that economists will always debate the merits of alternative strategies, such as the orphan drugs issue or the differential discounting debate, there is no one ‘right answer’ but a set of alternative strategies that society must choose from, HTA helps to inform that debate and highlight the advantages and disadvantages of each.

The challenge for HTA in Ireland, as Ciarán sees it, centres on capacity, infrastructure, and process clarity. In terms of capacity, there are a limited number of professionals working in the field, infrastructure required to gather the relevant information required for conducting HTA’s, and process clarity, in terms of visibility to the wider/non-HTA community of the process and the issues involved. “Events such as IPPOSI do help to address this”.



Industry Perspective

Based on a decade and more of experience in Industry on HTAs Jenny Hughes, Director, Vaccines & External Affairs, GSK, offered insights on the HTA process in a global pharmaceutical company and suggestions to improve the systems to improve patient outcomes and ensure continued industry investment into vital R&D. Jenny outlined her company's vision that "To be successful in the healthcare marketplace in the next few years it's all going to be about delivering value".

This value paradigm was explored and

explained. The role of the 'Reimbursable File' was highlighted as central to bridging the 'value gap' between what R&D provided in the past and What is needed now. Looking to the future Jenny proposed that "Incremental clinical benefit is a necessary, but not sufficient condition". Payers are increasingly analysing the:

- Unmet economic as well as clinical needs
- Incremental benefit relative to the payer's choice of comparator
- Economic (and social) significance of the clinical benefit
- Confidence that benefits will be replicated in real world practice
- Quality of evidence
- Extent of risk of inappropriate resource use and budget overspend

Jenny took the audience through some real life HTA processes from GSK including insights on how the following medicines fared in the process; Eltrombopag (ITP) and Pazopanib (RCC). The actual schema of a HTA was usefully shared with the audience to make the process more meaningful. A vision for an improved HTA process was explored. Its highlights centred on the following principles;

- HTAs should be based on early and inclusive dialogue, including with patients and
- Risk-sharing and flexibility is required in handling uncertainty
- Positive HTA outcomes should be implemented

- Time to market is more unpredictable, greater transparency on decision making post HTA decision is urgently required

A European Patient Perspective

Liuska Sanna, European Patients Forum (EPF), began by stressing the need for 'meaningful' patient involvement in the HTA process as a theme for her presentation. The EPF have identified both benefits and challenges that need to be capitalised on in the HTA process. Patients bring a qualitative value rather than a quantitative value to decision making. However, for patient involvement to be meaningful, measurability is important. How this can be achieved is yet to be defined perfectly in any EU state. Liuska reinforced previous speakers comments that HTA processes are unwieldy, mysterious and unclear for patients – the very stakeholder they are supposed to be focused upon! Unfortunately, this is creating a significant barrier to meaningful patient involvement in the process that must be urgently addressed.

Patient involvement can occur in 3 dimensions:

- Patient organisation representatives
- Lay patients
- Informal carers

The results of a recent EPF Survey 'Patient involvement in HTA in Europe' provided key signposts and learning's for the conference. Highlights included:

- Main interlocutors are patient organisations – majority of agencies indicated cooperation with average of 3 organisations, most operating at national level

Ms. Sanna noted that "there are clear differences of levels of patient involvement between the EU15 and the



EU12, partly due to a longer history of HTA in the EU15, but overall the level of involvement scored better than expected”

The survey of HTA agencies outlined optimal patient involvement practices which have been recommended:

- Public involvement unit within the agency
- A formal patient, carer and public involvement policy
- Offer payment for lay patient involvement
- Set up an advisory committee of lay people including patients
- Organise HTA events specially related to patient participation

The two major challenges identified from the survey centred on lack of capacity and a lack of patient knowledge of HTA, which need to be addressed.

Within the broad spectrum of patient involvement, Liuska also highlighted the need for the range of patient perspectives to be broadened, that is, beyond one or two patient groups, or individuals. HTA agencies or policy makers need to know the impact of patient organisations vs. Lay patient involvement in the decision making process. The patient perspective on resource allocation, location of resources (e.g. centres of excellence hospitals etc), and in terms of HTA competencies, patient organisations need training in HTA but conversely, HTA agencies need to train and develop their own perspective on patients involvement.

Commenting overall on the survey, Liuska noted that the responses had been mainly perception based; only 2 agencies had performed formal assessments of the patient involvement impact.

“A similar survey is to be conducted across EU decision makers however, identifying who these decision makers are in each of the countries is a struggle in itself”.



3. Summary of discussions

Eibhlin Mulroe, IPPOSI, opened the floor to discussion in the frame of three themes she had observed during the presentations:

- Transparency in the HTA process and following NCPE/HIQA recommendations
 - In terms of decision making
 - In terms of patient liaison
- The threat to R&D
 - Is the process at the end stifling R&D at the beginning?
- Patient training / capability building in HTA & access to information

Delegates to the conference posed several questions to the panel following the formal presentations. One key question centred on the practicality of how would they, the panellists, like to see patient involvement being developed in Ireland in the future?

Dr Ryan was keen to contend that from a HIQA perspective patient involvement is already a strong concern, “for every HTA an advisory group is established including (1) patient representative(s) in a specific/relevant disease area, and (2) “lay” representative(s) of the general population. The new clinical guidelines in development also include patient perspective considerations. Further, it is envisioned that stakeholder representative guidelines will be developed (HIQA & National Guidelines). A program advisory group, including patient representative(s), to advise HIQA on their processes and prioritisations is also planned.”

The panel were also asked, where a patient group exists it is easier, but what about patients who are not members of an organisation, or where no organisation exists?

Dr Ryan suggested that “HIQA have not previously involved ‘lay’ patients, this is partly due to the work HIQA have conducted to date, where patient groups have existed. It must be remembered that where capital spends of €15million and upward are involved, selection of appropriate patient representatives is important.” Ciarán added “In terms of the HTA process, patients are heavily involved in the development of quality of life instruments at the early stages of clinical trial development”.

Referring to the previous IPPOSI meeting held on this topic in 2008, and in particular the promising speech given by Ross Hathaway at that time, Fred Doherty, Genzyme, asked – “how is the hospital budget hurdle, particularly in the case of rare diseases, being addressed? In 2008 we were promised initiatives such as ‘the money follows the patient’ and ‘national clinical directorates’ however, there has been no National Clinical Directorates or ring-fenced budgets (for orphan diseases)? In attempting to follow up these issues the problem is either cited as a funding issue for the HSE or a policy issue for the DoHC, and the issue is bounced between the two.”

In response, Dr Ryan pointed out that “Mini-HTA’s are intended for hospital level decision support, it is hoped that this will help avoid situations where depending on which hospital you went to you could get access to the drug or not. Currently at hospital level no data is gathered in the hospitals to capture drug spend, other than top line figures. This is partly due to the current system of devolved budget control to the hospitals. The proposed ‘Mini-HTA’ system will standardise the HTA process across hospitals so that all hospitals will have a uniformity of access to medicines”.

“However, these plans are superseded by the current government’s plans to remodel the health system. Building to the Dutch system is a huge undertaking and will require time and resources to implement”. One proposal under these new changes is for a Patient Safety Authority to be set up”. Dr Ryan added “It is also worth noting that the Dutch system has a special Orphan Drug HTA process set up”.

While pharmaceutical companies appear to have a clear understanding of the HTA process in Ireland, the process for Medical Devices companies are not so clear, the panel were asked if they could offer advice to the medical devices sector of industry on this.

Dr Ryan commented that a national program for devices is currently being conducted by HIQA to address this issue, however, guidelines on medical device HTA’s were published by HIQA last year. “What are required are clearer decision pathways to be developed. One difficulty, comparing medical devices to the pharmaceutical situation, is that pharmaceuticals tend to stay static, in that they do not evolve or change over time, while medical devices constantly evolve and have many iterations. This makes

the lengthy process of HTA assessment of medical devices more difficult to remain relevant”.

Drawing on developments and experiences in the UK, Joe Kelly, Pfizer, asked “Is there plans to develop a reference cost model? Casemix and costing resources such as HIPE data are difficult to access; where are we and where do we need to be?”

Ciarán addressed this question, “In Northern Ireland, you can access reference costs right down to the individual level, this is where we need to be. There is progress across the board, but it depends on the research being conducted. Where there are gaps in the data, UK data can be adjusted to Ireland, but the process of data capture is still evolving for Ireland”. Dr Ryan added that next year HIQA intend to develop a strategy around data access.

As a closing statement, the panellists were asked to share a thought as to what they see as the way forward for HTA in Ireland.

Liuska saw the way forward involving better patient involvement around the HTA process and standardisation on how HTA is conducted. Jenny called for “different processes and frameworks for different interventions, for example, for a vaccine HTA a societal perspective would be more appropriate than a HSE perspective in conducting a HTA”. Ciarán noted the complexity of the issue, in that “the fact that different countries across Europe have different threshold levels is a concerning issue, it is also important to consider equity weights and how equity should be incorporated into our modelling”. Dr Ryan concluded that “standardisation should be important, but only where appropriate, overall our strategic priorities would be (1) stakeholder involvement and (2) cost data access. The debate around the threshold certainly needs to occur”.



Supported through an unrestricted educational grant