



IPPOSI

Irish Platform for Patients' Organisations,
Science and Industry

ACCESS TO MEDICINES AND NEW MEDICAL TECHNOLOGIES IN THE ERA OF HEALTH TECHNOLOGY ASSESSMENTS IN IRELAND



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1. THE REPORT AND ITS AIM

In January 2008, IPPOSI identified the issue of access to medicines/medical technologies and the role of Health Technology Assessments (HTAs)/Pharmacoeconomic Assessments (PEAs) in the decision making process as a key subject for Ireland to examine. For the future, important questions need to be asked and difficult decisions made. Ireland is not alone; these are issues that affect all countries. Given the timeliness for a discussion on this important subject, IPPOSI facilitated a conference on the 24th of November 2008 in the Croke Park Conference Centre (Dublin) attended by 150 delegates from State and Semi State Bodies, patient groups, clinicians, industry and academia. The aim of the conference “Access to Medicines and New Medical Technologies in the Era of Health Technology Assessments in Ireland” - to examine experience to date, explore the challenges and opportunities for the future together with key national and international experts, players and stakeholders. The conference also sought, as **Mr Godfrey Fletcher**, the new incoming IPPOSI Chairman and CEO of the Cystic Fibrosis Association of Ireland, said in his opening address, “to develop consensus recommendations for the way forward in Ireland ... to develop an approach which is relevant to us and to our unique health system”.

2. EXECUTIVE SUMMARY

The IPPOSI conference addressed three important issues: (1) Health Technology Assessments in Ireland, (2) Ireland’s Experience to Date, (3) Europe’s Experience to Date.

The HTA/PEA process in this country is about to change, new procedures and entities have been created and increasingly decisions will need to be taken on how to spend limited resources in the field of healthcare. HTA/PEA has a role to play in this decision making process. It should, **Professor Brendan Buckley**, European Centre for Clinical Trials in Rare Diseases (UCC) believes “form the scientific basis for making the right choices ...”.

The conference explored future changing structures for HTAs/PEAs in Ireland – examining the plans and how they will be developed in line with national and international practice. Representatives from four key Irish stakeholder groups (clinicians, industry, patient organisations, public health service (HSE)) shared their experience of HTAs/PEAs to date in this country and their hopes for the future under the new regime. Conference delegates also heard from a panel of international experts who explained how HTAs/PEAs are undertaken in the UK and in the Netherlands. They put forward what, in their opinion, Ireland might learn from international experience. Some of the key points raised include:

- Ireland needs to foster a consensus based approach to the development of HTAs/PEAs rather than a prescriptive approach.
- The scope of HTA/PEA 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/PEA system and to formally engage in the HTA/PEA process.





3. MANY ISSUES IN COMMON

Constant innovation in science and medicine bring challenges for health systems and HTAs/PEAs have increasingly emerged as a tool for informing more effective regulation of the utilisation and diffusion of health technologies. Opportunity costs exist for all decisions – where finances are limited, as they are for all healthcare systems, compromises and difficult decisions must and will be made. There is no disagreement – limited resources must be used wisely. Challenges around issues of affordability, coverage, universality, evidence, transparency, participation and redress; challenges not unique to Ireland, certainly exist.

Internationally, HTA/PEA is recognised as a relatively new and developing discipline. As with the international community, Ireland faces challenges in relation to a skills shortage in the area of technology assessments. Nationally, the absence of good cost databases and robust, reliable data for comparator and sensitivity analysis also prove challenging. Transparency of decision making processes and participation of key stakeholders including patient representatives and patient organisations are additional challenges. With limited budgets and depressing economic climates it is imperative that new health technologies are evaluated via a process that examines not only information about medical and economic consequences but also looks at the social and ethical issues in relation to the use of a health technology in an unbiased, robust and systematic manner.

A number of recommendations emerged from the day's discussion, the full list can be found in the body of the report. Briefly summarised some 3 general, but key, recommendations emerged:

Ireland needs to build capacity in the area of PEA and HTA.

- Strategies for enhancing communication and participation in the HTA/PEA process need to be continually refined and developed particularly in relation to patient group involvement.
- Extensive work needs to be done to improve the availability of high quality, nationally representative cost data to enhance the quality of the information available to the decision-makers.

4. FOREWORD – IRELAND'S HEALTH LANDSCAPE

Over the last decade innovative medicines and new health technologies have contributed to significant improvements in health status and quality of life. All across Europe people are living longer. In Ireland on average people are living into their seventies and eighties and, as a result, are spending a higher percentage of their lives in close contact with the health system. Treatments are also improving while becoming more expensive and resources to pay for such treatments are not increasing. These changes are putting our health system under increasing pressure. In fact, according to **Dr Michael Barry, Clinical Director, National Centre for Pharmacoeconomics (NCPE)** “over the last decade there has been a five fold increase in expenditures on medicines. In 2007 expenditure on medicines under the Community Drug Scheme was 1.74 billion euros”. It's not just the level of expenditure that is worrying it's the rate of the increase. “Year on year increase in expenditure on medicines in this country is amongst the highest in Europe” driven mainly by the fact that we are prescribing newer and more expensive medicines. This, coupled with the changes to the eligibility criteria for the drug schemes are having a noticeable impact. It's a complex situation of that there is no doubt. The global economic crisis which Ireland is also experiencing has put a renewed focus on value for money. Healthcare has always been complex but today its complexity is unprecedented. Indeed in Ireland, the challenge is, as **Mr Fred Doherty, External Affairs Director, Bristol-Myers Squibb** so aptly put it “compounded by the fact that the prescriber does not pay, the payer does not prescribe and the user is often unaware of the cost or the value of the medicine that has been prescribed”.



5. CURRENT HTA PROCESS IN IRELAND

Heretofore, there have been no systematic processes in Ireland to evaluate the clinical and cost effectiveness of health services. This is changing however; greater structure is being brought to bear on the process with greater use of PEA and HTAs in the future with the involvement of the NCPE and the new Health Information and Quality Authority (HIQA) which was set up in May 2007.

The National Centre for Pharmacoeconomics (NCPE)

Before the arrival of HIQA, the National Centre for Pharmacoeconomics (NCPE) advised the Department of Health and the Health Service Executive (HSE) on requests for cost effectiveness analyses (Cost/Life Year Gained (LYG)) in relation to medicines or cost – utility analyses (Cost/Quality Adjusted Life Year (QALY)). High cost medicines or those with potential significant budget impact on the Irish Healthcare System are subject to assessment, undertaken in accordance with the (2000) Irish Healthcare Technology Assessment Guidelines with decisions notified within 90 days in keeping with the 2006 Irish Pharmaceutical Healthcare Association IPHA/HSE Agreement.

As with other countries, Quality Adjusted Life Year (QALY) considerations are also applied in Ireland. In this jurisdiction, traditionally, drugs with an Incremented Cost Effectiveness Ratio (ICER) of less than 45,000 euros per QALY have been recommended for reimbursement. However this is not a fixed threshold, drugs with as high as €57,280 per QALY have been recommended for reimbursement. Be that as it may, cost effectiveness is a key consideration in PEA/HTA. When it comes to orphan drugs the use of such QALY thresholds are problematic and inappropriate. Orphan drugs are essential for rare disease patients and pose real challenges for the HTA/PEA process. During the question and answer session, Ms Siobhan Gaynor, Molecular Medicine Ireland (MMI) asked the panel how the cost of undertaking HTAs/PEAs by SMEs/academic groups in the rare disease and orphan drug/technologies and devices area and the expertise required to ensure the right data from clinical trials is collected would be addressed; as the cost implications for these bodies would be a huge hurdle. It was clear from the experts that there are no hard and fast answers. It is a challenge and attempts will have to be made to reward innovation and where appropriate, to reimburse above the set threshold.

NCPE has been undertaking PEA/HTA since 1998. Over the period January 2005 – November 2008 it has undertaken a total of 38 evaluations; 17 submissions were accepted without modification, 4 were accepted with modification and 17 (mainly price modulations) were rejected. Interestingly, the HPV vaccine was not introduced by the Health Minister, Mary Harney TD despite the positive HTA

recommendation. This highlights the point that despite the HTA/PEA outcome, the final decision regarding access to medicines rests with the Minister for Health and the Department.

There are examples in the past where HTA/PEA outcomes have been positive and resulted in a technology being made available. For example universal childhood vaccination programmes for pneumococcus and Hepatitis B. However, when finances are limited additional questions need to be asked – if the HTA/PEA produces a positive recommendation – is the impact on the budget, if the recommendation is implemented, affordable? Given the economic downturn being experienced in Ireland and globally, the Minister for Health felt it was not. During the conference, **Barry Andrews, TD, Minister for Children and Youth Affairs** referred back to the controversial HPV vaccine decision and reassured conference delegates that he was “quite determined and hopeful that we will certainly accept the findings of HIQA’s HTA sooner rather than later,. . . rather than wait for the recession to end. We do want to redress the issue in relation to cervical cancer vaccine” he said.




Minister Barry Andrews and Prof Moira O'Brien, Irish Osteoporosis Society

With limited and finite finances it will not be possible to keep adding product after product to the GMS without taking some away. Disinvestment is certain to feature in the future, Dr Barry warned. “Products that don’t work very well like clinical nutritional products for example” will need to be re-examined. “HTA/PEA submission requirements will become more sophisticated including probabilistic sensitivity analysis and acceptability curves. A move towards disinvestment guided by HTA/PEA is possible” he concluded.

The Health Information and Quality Authority (HIQA)

The Authority was established in May 2007 and one of its remits is to “evaluate the clinical and cost effectiveness of health technologies including drugs and to provide advice arising out of the evaluation to the Minister for Health and the Health Service Executive”. The aim - to speedily introduce technologies with significant health benefits, to



prevent the introduction of those technologies which fail to meet the requirements of evidence based analysis and to continuously monitor the effectiveness of technologies after their introduction. The remit – not dissimilar to that of the NCPE. For HIQA, the term “technology” is not limited to that of drugs, rather it includes any intervention that may be used to promote health, to prevent, diagnose or treat disease, or that are used in rehabilitation or long term care. This includes drugs (pharmaceuticals), devices, medical equipment, medical and surgical procedures, as well as the organisational and supportive systems within which healthcare is provided.

Since establishment, HIQA has undertaken two HTAs/PEAs; HPV vaccine and colorectal cancer screening programme. Given capacity issues, HIQA contracted both these HTAs/PEAs to external bodies but, **Dr Patricia Harrington, Acting Director for HTAs (HIQA)** revealed that the plan for the future is that at least 2/3rds of HTAs/PEAs will be conducted internally by the Authority. Where this will leave the NCPE is not clear and no concrete answers were available at the conference. Time, it appears, will tell.

Dr Harrington explained that the purpose of the HTA/PEA for the HPV vaccine was to “estimate the cost effectiveness of a combined national HPV vaccine programme and cervical screening programme compared to cervical screening alone. This was a limited HTA/PEA focusing on cost effectiveness alone”. The second HTA/PEA, one which is ongoing, with results expected in the first quarter of 2009, is a larger scale HTA/PEA looking at clinical and cost effectiveness, resource implications and ethical considerations related to the introduction of a population based colorectal cancer screening programme in Ireland.

Dr Harrington noted that HIQA has a statutory remit to provide advice to the Minister for Health and Children and the HSE. Highlighting the word “advice” she clarified that the purpose of HTA/PEA is to inform decision making, but that the HTAs/PEAs do not represent the decisions in themselves. Many factors other than cost-effectiveness may impact on a decision including equity and societal considerations, overall budget impact, and the relevance of the technology in the context of the overall healthcare service. For example, despite a positive HTA/PEA recommendation, the HPV vaccine has not been immediately introduced by the Health Minister, Mary Harney TD. This highlights the distinction between the HTA/PEA outcome and the decision-making process. The final decision regarding the adoption of a technology rests with the Minister for Health and Children and the HSE.

So what are the objectives and future plans for HTA/PEA in Ireland? Well HIQA plans first to establish a quality framework for the conduct of HTA/PEA. In other words to establish where HIQA stands in relation to HTA/PEA, how the Authority will be governed and how it will take advice in relation to HTA/PEA. Two key advisory groups will play an

important role; (1) Programme Advisory Group, (2) Scientific Advisory Group. The Scientific Advisory Group, established in May 2008 provides the Authority with the methodological expertise in the conduct of HTA/PEA. The group, comprised of national and international HTA/PEA experts, stakeholders across the healthcare setting including academics, healthcare policy specialists and patient representatives, is currently working on national guidelines to “guide the use and development of HTA/PEA nationally in Ireland” Dr Harrington confirmed. The Programme Advisory Group, which is currently being established will assist the Authority in terms of the prioritisation of HTA/PEA and will also advise HIQA in relation to its interaction with stakeholders. There will be a broad representation across the healthcare setting in this group. Expected priority areas for HTA/PEA will be chronic conditions, cancer and conditions that have high budget impact or where there are significant safety concerns or concerns in relation to equity of access.

There are 3 broad levels of HTA/PEA for Ireland:

- (1) HTAs/PEAs of national significance will be conducted by the Authority. They will typically involve the use of independent economic models and will normally take anywhere from 6 - 18 months to complete (HPV vaccine and colorectal cancer screening programmes are examples).
- (2) Rapid/Single Technology HTA/PEA undertaken by the NCPE under the auspices of the agreement between the HSE and IPHA with regard to cost effectiveness – reimbursement of new pharmaceuticals under the Community Drug Scheme. The process takes 90 days and involves a review of company submissions as opposed to the use of an independent economic model.
- (3) Promotion and adoption of HTA/PEA techniques and HTA/PEA decision making to inform local level decisions at an individual level or community care level. These are undertaken at a local level (e.g. the HSE, individual hospitals, health plans). To assist this process HIQA has worked with the HSE to customise a mini HTA tool kit that could be used at local levels. This tool kit was piloted in an assessment undertaken by a multi-disciplinary team compiled by the HSE in relation to a Cystic Fibrosis Therapy Vest.

A number of key challenges clearly exist including a notable skill shortage in the area of HTA/PEA, widespread myths and misunderstandings surrounding HTAs/PEAs, and difficulties in obtaining accurate, generalisable data in relation to healthcare costs and usage.

6. RECOMMENDATIONS FOR THE FUTURE

The following emerged as key proposals for action:

- Clarity is needed with regard to the role and purpose of HTA/PEA in Ireland. The roles of NCPE and HIQA must be clarified. Dialogue and consultation must be part of the decision making process in establishing how HTA/PEA in Ireland should move forward.
- Ireland must invest in terms of personnel and strategies for PEA/HTA; to target and entice appropriately qualified people into this expanding and increasingly important field of expertise.
- HTA/PEA bodies must continue to ensure that clinical experts involved in Expert Advisory Groups for HTA/PEA have both the relevant specialisation AND the appropriate level of expertise.
- Stakeholder participation, communication of HTA/PEA results, the sharing of HTA/PEA reports and opportunity for redress are essential and must be available for all HTAs/PEAs including Mini PEA/HTAs¹.
- The development of national databases and registries, electronic health records and unique patient identifiers must be supported since the absence of good cost databases and quality direct information is so problematic and is impacting negatively on HTA/PEA. Dialogue around the current proposed Health Information Bill must continue so that these valuable resources can be developed.
- Ireland must develop a strategy to address the inevitable challenges (especially in cost and competency) HTA/PEA will pose for SMEs/Academic groups in the rare disease and orphan drug/technologies and devices field. There must be a different approach to Orphan Drug Products.

¹ The results of the mini HTA/PEA for the Cystic Fibrosis Physiotherapy Vest were not made public and some key stakeholders were not involved in the process. The Cystic Fibrosis Association of Ireland was informed afterwards that the technology being reviewed (eg. The CF Vest) would not add any benefit to actual treatment but the Association has not seen a copy of the report. HIQA noted that the aim of mini HTAs is to support decision making at local level and within the HSE. Neither the report nor the process is owned by the Authority, but one in which it may assist as part of a multi-disciplinary team. HTA undertaken by HIQA HTA Directorate will be made freely available on its website and information on the process will be open and transparent but HIQA cannot confirm that this will be the case for Mini HTAs since the Authority is not responsible for the process in these instances.

7. THE STAKEHOLDER EXPERIENCE OF HTA/PEA IN IRELAND

A Clinician's Perspective

Professor John Crown, Consultant Medical Oncologist, St Vincent's Hospital expressed a concern that the proposals for the future of HTA/PEA in Ireland could result in a system closely resembling the UK National Institute for Health and Clinical Excellence (NICE) model where "access to an entire portfolio of new technologies has been denied to UK patients or at best, delayed to them as a result". While rationing is inevitable in all systems, a NICE type approach would not, Professor Crown warned, be one Ireland should attempt to replicate for a number of key and obvious reasons; (a) the demographic in Ireland is very different to that of the UK, (b) there is a two tier healthcare system in Ireland and (c) in this jurisdiction the most vocal, most socially and politically influential have private health insurance. "Historically we have had good access to cancer drugs in this country ... the Regulatory Commission was the only central hurdle other than Hospital Formulary Committees and also a process for General Medical Scheme (GMS) which I believe is appropriate because the GMS does act within a finite budget" Professor Crown told delegates. With the increased use of HTAs/PEAs extra hurdles will be introduced and drugs which have been available and which have enhanced wellbeing and quality of life could be denied our public patients, he cautioned. Dr Michael Barry in intervening did reassure the audience that, in contrast to England and Wales, no licensed cancer drug had ever been denied to a patient in Ireland because of a HTA/PEA.

An Industry Perspective

Mr John McCarthy, Partnerships Director, Pfizer Healthcare Ireland told conference delegates that Pfizer's experience of the HTA/PEA process in Ireland has been "almost universally positive both in the HTA/PEA process and in assessment for reimbursement". Citing inhaled insulin as an example of the organisation's positive experience, John told the gathering that the HTA/PEA was handled within 8-9 weeks. "It was so encouraging to see that such a complex submission could be handled in such a timeframe, taking on board patient welfare and the innovation of the medicine. The process was consultative and transparent ... fair and valued innovation". While not all of the model was accepted "there was a clear understanding of need, it had clinical relevance and it was for patient benefit". A number of factors contributed to this successful engagement including Pfizer's willingness and ability to actively participate in the process. The organisation could draw on its corporate capabilities from its Northern European affiliates who have a much stronger experience of HTA/PEA. The organisation also made a conscious and deliberate decision to share horizon and pipeline views and this has, John feels been the right choice. In preparation for the future, Pfizer is in the process of developing internal capacity in the PEA/HTA area so that the organisation is better placed to engage more actively in the HTA/PEA processes.



Mr Ross Hattaway, Corporate Pharmaceutical Unit, HSE and Mr Jim Reilly, Patient Focus

The Health Service Executive (HSE) Perspective

The Corporate Pharmaceutical Unit, HSE is the unit responsible for providing a reimbursement approval process for new products for community use. Value for money is a key reimbursement consideration for the HSE, especially now given the tight economic climate. IPHA/HSE and other agreements with industry and manufacturers are an important step and **Mr Ross Hattaway, Manager, Corporate Pharmaceutical Unit, HSE** explained that the hope is that the “2006 agreements will be the first of 4-5 four-year agreements which will enable the HSE to provide a level of certainty with regard to the general direction of how drugs will be reimbursed in Ireland over the next 20 years”. The HSE also wants to give certainty for products coming off patent, providing “clear structures for how they will be priced” so that when a product comes off patent it will have the opportunity to “compete fairly and effectively as a branded generic in the Irish market”. The reimbursement process, he explained “is quite straightforward” something **Mr Larry Warren, CEO Alpha1** could confirm when he addressed the delegates. “We are lucky in Ireland” Larry said “our system is actually one of the simplest and easiest to understand”. The HSE reimbursement procedure is based on a number of key guiding principles; affordability, availability, continuity and security of supply and value for money. In addition, set criteria exist which, under the EU Transparency Directives, the HSE must notify to the European Commission re pricing and any industry arrangements. The process is transparent and efficient since IPHA and other Agreements provide administrative simplicity for the HSE. As with other speakers Ross explained how access to reliable data on price is a limiting factor and poses a real challenge for the Executive. He reminded delegates that extended PEA/HTA “are triggered by budget impact and by clinical innovation and benefit for patients. There are no hard and fast rules on when we seek PEA/HTA however when you have to decide whether a treatment is more effective than existing treatments you must be in a position to make that decision”; information on price, effectiveness and impact on quality of life all influence that decision. That said, the HSE would expect only a dozen PEA/HTAs annually as for a lot of products it is clear that there is no requirement for this since there is a proven track record for similar types of therapies, they look to be equivalent in price and use and are

not going to increase the budget. “We are at arms length from the assessment process – we are not directly involved in this process and nor should we be”. Unlike the UK NICE system, Ross reminded participants that Ireland does not have universal eligibility and so decisions relating to reimbursing and supplying products is very different. Pricing discussions, of which there have been a number, are also another key difference. “Where we want to reimburse but cannot afford to do so at the given price we have to find a price where it is affordable for the supplier and ourselves”. This is where the benefit of early engagement, dialogue and contact play important roles. Increasingly also companies are approaching the HSE indicating that they will be introducing a product and seeking advice regarding next steps. In such instances the HSE is in the position to suggest that industry dialogue with the NCPE to establish if the experts there think a PEA/HTA will be required. If a PEA/HTA is likely, industry can start preparing for that and it can be done in parallel with the licensing process. “In our view the process has been successful and it is a model that should be built on” Ross told delegates. The process is “flexible, independent, transparent, evidence based, with significant supplier involvement, a 90 day decision period and an appeals process” – these are the key features of the HSE reimbursement procedure. In the future the HSE hopes to look at “line extensions, effectiveness, cross over products which need to be administered in a clinical environment but probably not in a hospital, unlicensed medicines as we are using these and paying for them. We will also be putting in place a pricing and approval structure for unlicensed medicines in the new year. Whether a product is licensed or not we have to know whether it is worth using and what we should be paying for it. In responding to a query from Niall McLoughlin (CEO, Irish Osteoporosis Society) regarding patient involvement in HSE reimbursement decisions, Ross said that the HSE is now looking at other models to see how this issue might be tackled. “Of interest is the Scottish Medicines Consortium which has at least one panel which involves stakeholders, patients, prescribers etc in an advisory role. The HSE does not see the process as a static one and as we have resources and information we will include better ideas such as patient involvement” he said.

A Patient Organisation's Perspective

The patient experience of access to medicines over the years has generally been very good. **Mr Jim Reilly, Development Officer, Patient Focus** said that “medicines have generally been freely available be it privately, through the GMS or the drug payment or hardship schemes”. The objective of HTA/PEA should be to ensure that resources deliver the best outcome for patients. Patients should be central to everything that is done in healthcare; ensuring that the patient voice is heard and considered is key to that process. Patient representatives are included in all HTA/PEA Expert Advisory Groups compiled by HIQA. Mr Stephen McMahon, Irish Patients' Association is a member of the HIQA Scientific Advisory Group and Sheila O'Connor of Patient Focus is also a member of a new group

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established by the Minister for Health to recommend efficiencies and savings. The recent HPV vaccine decision has however set alarm bells ringing and questions are now being asked around access and restrictions; will we have a NICE experience and will drugs going forward be denied on cost grounds? “Is what is being done, being done for the benefit of patients or the benefit of the exchequer?” Jim asked. “Patient advocates are vital to the welfare of the health system and those in it. Patients must be free to question without fearing that their treatment will be compromised as a result of calling the system into question”.



Dr Michael Barry, National Centre for Pharmacoeconomics (NCPE), Mr John McCormack, Irish Cancer Society, Dr Wim Goettsch, Healthcare Insurance Board (CVZ) and Prof Peter Littlejohns, National Institute for Health and Clinical Excellence (NICE).

8. THE STAKEHOLDER EXPERIENCE OF HTA/PEA IN EUROPE

The UK NICE Experience

Ten years ago in the UK there was widespread diversity in the ability of individual patients to get access to drugs particularly cancer drugs. The very profound problem resulted in postcode drugs lottery with great disparities in access, sometimes in adjoining geographic areas.

To address this problem, the National Institute for Health and Clinical Excellence (NICE) was set up to provide national standards on what good practice should be. As an evidence based organisation, one of the Institute’s remit is to provide guidance on the use of new and existing medicines, treatments and procedures within the NHS; “guidance based on a number of principles eg best available evidence, inclusivity, transparency, independence with a focus on cost effectiveness ...”. The guidance is issued direct to the NHS.


A review was undertaken in 2008 as the NHS celebrated its 60th birthday. The outcome report “High Quality Care for All” – otherwise known as the Darzi Report provided the overall strategy for the NHS for the coming years. Addressing delegates Professor Peter Littlejohns, Clinical and Public Health Director, NICE said that while the review was “very supportive of NICE” change was also heralded; “... NICE’s role in providing evidence would be expanding”. The appraisal programme would, going forward, include in its remit new

devices, new diagnostics and virtually all new significant cancer drugs. NICE has also “taken over the establishment of standards within the NHS so in the future any national standard will be based on NICE’s assessment of cost effectiveness to the appropriate clinical practice”. Over the next three years the NICE budget and staff will both increase (from £30 to £90 million and its core staff of 270 will increase to 500). A new programme around NHS evidence will also be created “to take all available evidence bases that are accessible to members of the professions within the UK and quality assure the process using a system of Advisory Committees”. Using modern technology the evidence will be made available on a daily basis. Recognising the importance of research evidence and acknowledging that increasingly innovations are being evaluated with very immature evidence bases, NICE is keen to harness patient experience and clinical practice knowledge so that this can feed into and inform the NICE Advisory Committees. To ensure transparency these committees now meet in public. There is also a Citizens Council that looks at broad strategic questions.

Disinvestment is an important feature and Professor Littlejohns confirmed that his “group within NICE is responsible for publishing four disinvestment recommendations every month”. For NICE the question is not should the decisions be made, the question is how they are made and whether or not they are made in an open and transparent manner. NICE also recognises that fairness, openness and transparency is perspective dependent and it is never going to be possible to suit everyone. Over a four year period (April 04 – August 08) NICE appraised a “total of 39 cancer indication pairs ... recommended 15 without any restriction, 14 were restricted to certain population groups and 1 was recommended for research only”. As with other jurisdictions access to information and evidence base is also problematic and challenging for the UK. Like other jurisdictions, once a decision is made which does not meet with stakeholder approval; public fury and outrage erupts. The message is simple, no country can meet all the demands for healthcare that a technology-driven health service can generate. Difficult though it may be there is a need and a place for economic appraisals. Restrictions based on cost effectiveness and affordability are a reality in the 21st century health care system, we must acknowledge and accept this, what is important is that the decisions are overt. HTAs/PEAs are here to stay.

The Dutch Experience

Dr Wim Goettsch, Consultant Pharmacoeconomics, Dutch Healthcare Insurance Board indicated that healthcare and HTAs/PEAs should not boil down to pure economics. Social, ethical, legal concerns, claims for solidarity are also important. Cost effectiveness is important but should not be the only consideration - drug effectiveness and relative effectiveness of technologies compared to current standards should be the first major and most important concern. Addressing the conference Dr Goettsch told



delegates that “everyone in the Netherlands is privately insured”². In deciding whether or not a health technology should be included in the basket of insured healthcare interventions, the Netherlands adopts a two phased approach; (1) an assessment phase and (2) an appraisal phase. In the Assessment Phase information on necessity, effectiveness, cost effectiveness and feasibility are examined. In the Appraisal phase the results of the assessment are balanced re the legal, social and ethical arguments for or against the introduction of a health technology. Cost effectiveness only becomes relevant if there is a therapeutic added value that will imply additional cost. “Two elements are essential in the valuation of cost effectiveness results – robustness, quality of data and the outcome in incremental costs per quality adjusted life year (ICER)”. Robustness of pharmacoeconomic data is essential but like other European countries the quality of available data is problematic. “In most countries the manufacturer is responsible for PEA/HTA ... it is comparable to a single technology appraisal in which a company is responsible for an assessment .. as a result there is still a lot of debate regarding the analysis and the quality of the analysis”. Without a QALY threshold an ICER is difficult to interpret but despite this the Dutch believe that thresholds should not be fixed because arguments like severity of disease or rarity of disease are important considerations and need to be taken into account in reimbursement decisions. The Dutch, like the Swedes and the Belgians have therefore decided not to use a fixed threshold. The key message - assessments are important. Necessity, effectiveness, cost effectiveness and feasibility are key considerations but cost effectiveness considerations can only be effective if reliable, accurate and structured, robust data upon which to make decisions is available. Thresholds should not be fixed, flexibility and ranges are much more appropriate. Healthcare reimbursement must not be reduced to an economic argument. Social, ethical and legal considerations are essential also.

9. KEY STAKEHOLDER RECOMMENDATIONS:

- Ireland needs to foster a consensus based approach to the development of HTA/PEA.
- HTA/PEA in Ireland should not be reduced to an economics argument; social, ethical and legal considerations are equally important.
- Ireland must ensure that multi annual budgeting is introduced in healthcare as an important part of reform. Central and key to this process is industry willingness to share pipeline and horizon views.
- Industry needs to engage in the HTA/PEA process from its earliest stages across the whole PEA/HTA chain from discussions around the new technology, the kind of evidence that will be required, what comparative treatments the technology will be examined against etc. Early price discussions in the reimbursement process are also imperative.
- Industry submitting the drug or device for PEA/HTA must have the capacity to engage in the process and discussion.
- Europe needs to look at the system of mutual recognition for reimbursement (where one therapy/technology is recognised in one country and reimbursed it should be recognised in another and reimbursed) and EU Member States need to engage actively in this particularly in the case of Orphan Drugs for Rare Diseases.
- QALY and how it is assessed and measured should incorporate the true patient experience in the 21st century including for rare disease patients.
- Patients and patient organisations should be assisted and empowered to better understand the HTA/PEA system and to formally engage in the HTA/PEA process.

² A part of the funding for this is collected through taxes and in addition every citizen pays 100 euros a month. Children up to 18 do not pay and for those on low incomes there is a tax refund system.



ICAB	11.48	+14
NwPerso	16.17	+02
WashB	28.66	+13
Amor Skar	21.68	+24
JanCar	29.06	+19
Express Print	5.91	+06
Express Y	24.42	+09

10. AFTERWORD

Patient involvement in healthcare decision making is crucial. NICE has increasingly recognised this. Ireland needs to ensure that as we move forward that crucial voice does not go unheard. Patient needs and welfare must be at the centre. This was the message coming from many quarters. **Mr Rod Mitchell, Chrons, UK, EPPSI and European Federation for Chrons Disease** called on the Irish patient organisations to “get involved as much as you can”. **Mr John McCormack, CEO, Irish Cancer Society** agreed and reminded delegates that the patient voice is crucial. “Patient organisations in this country are fighting important battles” for services and “have engaged in health service reorganisation debate” he said. This is a view and a voice that needs to be heard. Professor Littlejohns also conceded that the patient voice was important and in responding in the question and answer session told delegates that patients and the public are able to engage in all NICE guidance production. Patient advocacy groups are invited to comment on the guidance. Patients are also involved in the Advisory Committees and in the Citizen Council. NICE also has a Patient and Public Involvement Programme. A number of patient surveys have also been undertaken.

11. AND FINALLY








The last word of the day was left to **Ms Eibhlin Mulroe, CEO, IPPOSI** who told delegates in preparing for the meeting that she had spoken to Mr Eric Low, CEO Myeloma UK who made a very thought provoking point. “Imagine” he said “finding yourself on top of a car roof trying to escape flood waters because a river has burst its banks. If you are not rescued, death is imminent. In such scenarios millions of pounds can be found to rescue this one person. It won’t matter that that person could actually be a myeloma patient with only months to live or someone who has had a number of heart attacks. It is just someone who is in a near death situation through no fault of their own with a television camera filming as events unfold. If it costs £5 million to rescue that person we will do it because there is a value attached to that because of the rule of rescue”. This, Eibhlin said had made her think and question why “rescuing” someone eg rare disease patient in need of expensive medicines and health technologies should be any different. “We must engage and work with all stakeholders, this is the only way we will move forward positively. We need to look at all aspects not just the economic arguments, although important, there are ethical arguments of great importance to take into account”.



Ms Gina Plunkett and Ms Christina Donnelly, Irish Chronic Pain Association with Mr Larry Warren, Alpha1 Foundation.



SOME KEY RECOMMENDATIONS :

-  **Ireland needs to foster a consensus based approach to the development of HTAs/PEAs rather than a prescriptive approach.**
-  **Ireland must invest in terms of personnel and strategies for PEA/HTA; to target and entice appropriately qualified people into this expanding and increasingly important field of expertise.**
-  **Extensive work needs to be done to improve the availability of high quality, nationally representative cost data to enhance the quality of the information available to the decision-makers.**
-  **The scope of HTA/PEA should not be limited to economic considerations only. Social, ethical, legal considerations are equally important.**
-  **Industry needs to engage in the HTA/PEA process from its earliest stages across the whole PEA/HTA chain from discussions around the new technology, the kind of evidence that will be required, what comparative treatments the technology will be examined against etc. Early price discussions in the reimbursement process are also imperative.**
-  **Strategies for enhancing communication and participation in the HTA/PEA process need to be continually refined and developed particularly in relation to patient group involvement.**
-  **Patients and patient organisations should be assisted and empowered to better understand the HTA/PEA system and to formally engage in the HTA/PEA process.**