WORKSHOP

What is the patient's role in informing the decision process for approval and reimbursement of new medicines?

7-8 OCTOBER 2015

WINDSOR, UK

A SYNOPSIS

CIRS CENTRE FOR INNOVATION IN REGULATORY SCIENCE

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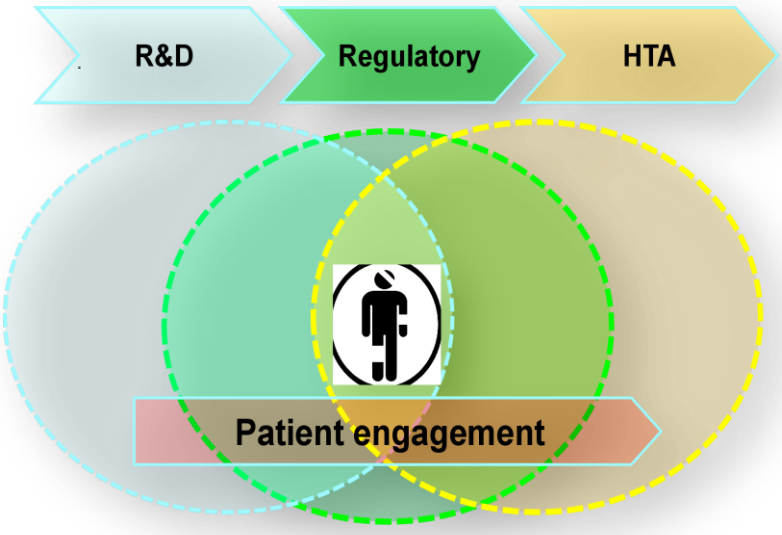
BACKGROUND

The patient's role in the development, regulation and health technology assessment processes for new medicines continues to grow in importance to all stakeholders and incorporating the patient perspective is now regarded as essential by many decision makers. Indeed as patient-centered care becomes more embedded into healthcare systems, the patient's perspective and clear identification by decision makers of the benefits, risks, values, and tradeoffs that are important to patients will be critical to making informed decisions.

Despite this awareness, and although methodologies for eliciting patient views throughout the product life cycle have been developed for some disease areas, concerns remain regarding issues such as the identification of representative patients and the duplication of efforts by industry and multiple agencies among the same groups of patients. Therefore, should an understanding of the patient's perspective be a continuum throughout the journey to bring new medicines to market?

This Workshop built on CIRS Workshops on this topic conducted during 2012-2014 and focussed on the current processes and procedures as well as the similarities and differences in approaches and expectations between the three key stakeholders (industry, regulators and HTA) to elicit patient input and whether there can be a way of simply collecting patients' views that can enable the patient's perspective to inform company and agency decision making.



The patient's role in the development, regulation and health technology assessment processes for new medicines continues to grow in importance to all stakeholders

The key questions for discussion were:
What are the learnings from current practices for patient involvement from different HTA and regulatory agencies?
How can industry and agencies identify truly representative patient viewpoints?
How do reviewers use the various inputs from patients and what weight do patients' perspectives have on the final regulatory or HTA decision?

WORKSHOP OBJECTIVES

- **Improve understanding** of the importance and value of patient involvement
- **Identify** best practices in the acquisition of patient input into the decision-making process
- **Recommend methods** for leveraging the same patient input for industry, regulatory review and health technology assessment

WORKSHOP CHAIRS

Prof Trevor Jones, *Chairman, Simbec-Orion Group, UK*

Prof Adrian Towse, *Director, Office of Health Economics*

INTRODUCTION

CIRS Executive Director **Lawrence Liberti** welcomed participants to the most highly attended CIRS Workshop to date, saying that the number of attendees would afford the opportunity to more fully understand patients' diverse opinions and perspectives, because as Hippocrates explained over 2800 years ago, "It is more important to know what sort of person has a disease than to know what sort of disease a person has."

Day one Workshop Chair, ***Prof Trevor Jones,*** *Chairman, Simbec-Orion Group, UK* set the scene for the meeting by recalling that several decades ago, HIV-positive patients subverted the processes of randomised clinical trials in order to force a conversation around how they could better inform their own decisions about the risks that they would take in their treatment. Today, industry and regulatory and health technology assessment agencies must use the knowledge gained from that experience to engage patients in partnerships that respect the integrity of all stakeholders.

WORKSHOP PROGRAMME

SESSION: THE CURRENT PATIENT ENGAGEMENT LANDSCAPE: A LIFECYCLE APPROACH CHALLENGES AND OPPORTUNITIES	
Chair's welcome and introduction	**Prof Trevor Jones,** *Chairman, Simbec-Orion Group, UK*
Key outcomes and recommendations from previous CIRS patient Workshops	**Dr Neil McAuslane**, *Director, CIRS*
Keynote presentation – Begin with the end in mind **Aligned patient engagement in the development, approval and reimbursement of new medicines - the key to ensuring medicines meet patients' needs and ensure value to the healthcare systems?**	**Nicola Bedlington,** *Secretary General, European Patients Forum*
Integrated patient involvement in the lifecycle of a new medicine: Industry viewpoint	**Dr Anton Hoos,** *Head of Medical Europe, Amgen, Switzerland*
The regulatory agency patient/citizen engagement landscape: Challenges and opportunities	
EMA approach	**Dr Isabelle Moulon**, *Head of Patients and Healthcare Professionals Department, Stakeholder and Communication Division, European Medicines Agency*
Swissmedic approach	**Dr Petra Dörr**, *Head of Communication and Networking, Deputy Director, Swissmedic*
The HTA agency patient/citizen engagement landscape: Challenges and opportunities	
***How do patients view the approaches by regulatory agencies for engagement?**	**Patricia Furlong,** President, Parent Project Muscular Dystrophy, USA
TLV approach	**Niklas Hedberg,** *Chief Pharmacist, TLV*
CADTH approach	**Dr Brian O'Rourke,** *President and Chief Executive Office, CADTHr*
Patient reported/relevant outcomes, patient preferences and patient perspectives: What are the main challenges to measuring/collecting and utilising the information?	
Industry viewpoint	**Dr Indranil Bagchi**, Vice President and Head. Payer Insights and Access, Global Health and Value, Pfizer Inc, USA
HTA agency viewpoint	**Dr Roisin Adams,** Deputy Head, National Centre for Pharmacoeconomics, Ireland
How do reviewers use various inputs, direct and indirect, from patients/citizens in their assessments and what weight or influences do these perspectives have on the final decision?	
Regulatory perspective	**Dr Susan Morgan,** Medical Assessor, MHRA, UK
HTA assessment perspective –	**Andrew Mitchell,** Strategic Adviser, Evaluation, Department of Health, Australia
Quality standards for patient/citizen involvement in HTA engagement	**Dr Karen Facey,** Evidence Based Health Policy Consultant, UK
Patient engagement – Industry case study –	**Dr Simon Fifer,** Manager, Research Development, Institute for Choice, University of South Australia

***Unable to attend**

Syndicate discussions	
Syndicate A: What would the optimal process of integrating patient engagement from development to reimbursement decisions look like?	**Chair: Barbara Sabourin**, *Director General, Therapeutic Products Directorate, Health Canada* **Rapporteur:** *Dr Michael Happich, Director, HTA BioMeds Canada & Europe, Eli Lilly & Co, Germany*
Syndicate B: **Measuring the impact and influence of patient/citizen input has had on the final regulatory and HTA decision – What would be the key components and measures? HTA perspective**	**Chair: Niklas Hedberg,** *Chief Pharmacist, TLV* **Rapporteur:** *Mikkel Sachs, Industrial PhD Student, University of Copenhagen, Denmark*
Syndicate C: **Measuring the impact and influence of patient/citizen input has had on the final regulatory and HTA decision – What would be the key components and measures? Regulatory perspective**	**Chair: Prof Robert Peterson,** *Executive Director, Drug Safety Effectiveness Network, Canadian Institute of Health Research* **Rapporteur: Dr Pieter Stolk**, *Project Manager, Escher, TI Pharma & Utrecht University, The Netherlands*
Syndicate D: How can HTA and regulatory agencies better meet the needs of patients by ensuring the patient perspective is held paramount?	**Chair: Alastair Kent,** *Director, Genetic Alliance, UK* **Rapporteur: Dr Paul Robinson,** *Executive Director, Patient Perspective, Merck, Sharp & Dohme, UK*
SESSION: INTEGRATED PATIENT ENGAGEMENT FROM BENCH TO REIMBURSEMENT DECISION	
Chair's introduction	**Prof Adrian Towse,** Director, Office of Health Economics
Patient engagement in the decision-making process – How do other stakeholders perceive their role?	
Policy perspective European commission	**Sevala Malkic,** *Policy Officer, European Commission, Belgium*
Society perspective	**Prof Dr Irina Cleemput,** *Senior Health Economist, Belgian Health Care Knowledge Centre (KCE), Belgium*
Clinician perspective	**Julian Walker,** *Director of Research and Development, Consultant Clinical Psychologist, Avon and Wiltshire Mental Health Partnership NHS Trust, UK*
Future perspectives – Looking forward to 2020: How do the different stakeholders see the patient engagement landscape and the role of the patient evolving to support their decision-making processes?	
Industry vision	**Dr Isabelle Stoeckert,** *Vice President, Head Global Regulatory Affairs, EU CAN Pharma & EU Consumer Care, Bayer Pharma AG, Germany*
Regulatory vision	**Dr Tomas Salmonson**, *Chair, CHMP, EMA*
HTA vision	**Hedi Livingstone,** *Senior Public Involvement Adviser, National Institute for Health and Care Excellence, UK*
Patients' vision	**François Houÿez**, *Director, Treatment Information & Access, European Organisation for Rare Diseases (EURORDIS)*

SYNDICATE SESSION RECOMMENDATIONS

Topic A: What would the optimal process of integrating patient engagement from development to reimbursement decisions look like?

Chair: Barbara Sabourin, *Director General, Therapeutic Products Directorate, Health Canada;* **Rapporteur:** *Dr Michael Happich, Director, HTA BioMeds Canada & Europe, Eli Lilly & Co, Germany*

- Clear definitions and framework are needed; build on the HTAi values and standards activities such that these can be further promoted and implemented by HTAs and regulatory authorities
- Work globally with regulators and reviewers to make the patient voice a more integrative part of their processes; promote FDA guidance on patient preference methodology; CIRS to foster collaboration with emerging regulatory and HTA authorities to encourage patient participation
- All stakeholders should investigate more comprehensive ways to collaborate and pre-specify with regulatory and HTA authorities regarding the integration of patient preferences in drug development to increase credibility, validity and acceptability of clinical data

Topic B: Measuring the impact and influence of patient/citizen input on the final regulatory and HTA decision - What would be the key components and measures? HTA perspective

Chair: Niklas Hedberg, *Chief Pharmacist, TLV;* **Rapporteur:** *Mikkel Sachs, Industrial PhD Student, University of Copenhagen, Denmark*

- Transition patient participation to a partnership in order to encompass a view of not only how to inform final decisions but to also respect and understand the decision process
- Sponsor and facilitate patient engagement research and establish standards of quality for patient involvement to maximise its impact
- Conduct a two-step evaluation of patient interaction: 1) evaluate interaction against established standards; 2) survey HTA agencies to determine if they were influenced and patients to determine if their voices were heard

Topic C: Measuring the impact and influence of patient/citizen input on the final regulatory and HTA decision – What would be the key components and measures? Regulatory perspective

Chair: Prof Robert Peterson, *Executive Director, Drug Safety Effectiveness Network, Canadian Institute of Health Research;* **Rapporteur: Dr Pieter Stolk**, *Project Manager, Escher, TI Pharma & Utrecht University, The Netherlands*

- CIRS should map and identify gaps in current global frameworks for patient engagement throughout the product lifecycle
- CIRS could survey regulatory authorities globally on their current patient engagement activities , including the post-approval phase
- There should be an initiative to develop evaluation measures for patient engagement in the regulatory process, including an assessment of work that has been done; the outcomes could be presented potentially as part of a future CIRS Workshop
- Consider for a future topic: how does the involvement of patients in the regulatory process impact patient decisions in clinical use and overall disease management?

Topic D: How can HTA and regulatory agencies better meet the needs of patients by ensuring the patient perspective is held paramount?

Chair: Alastair Kent, *Director, Genetic Alliance, UK;* **Rapporteur: Dr Paul Robinson,** *Executive Director, Patient Perspective, Merck, Sharp & Dohme, UK*

- CIRS could review existing institutional guidelines for conflict of interest, summarise and make recommendations
- A consortium could be organised to review methodologies for patient engagement and examples from companies, regulators, summarise and share best practices for use of a scientific approach
- In recognition of practical limitations to agency and patient capacity, the purpose of public engagement should be clarified to ensure effective and efficient interactions.
- Assess the transferability of patient perspective across countries, particularly so that smaller countries can benefit from the experience of others

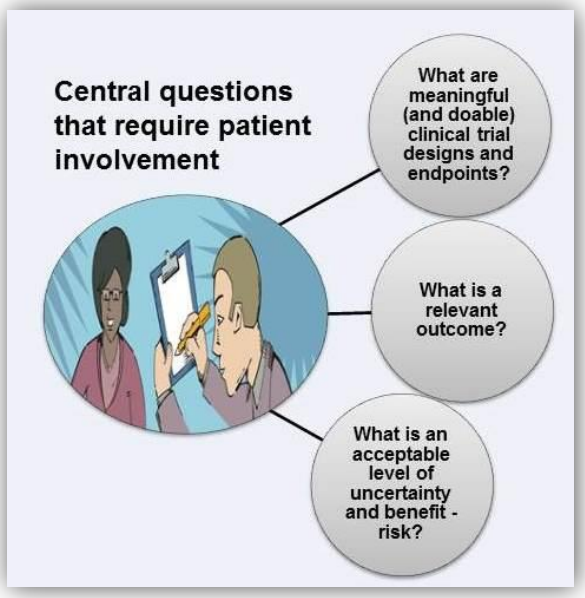
PRESENTATION HIGHLIGHTS

THE CURRENT PATIENT ENGAGEMENT LANDSCAPE: A LIFECYCLE APPROACH - CHALLENGES AND OPPORTUNITIES

Recent CIRS Workshops have documented progress in patient involvement in the lifecycle of new medicines, reflected in the successes of industry- and patient-led initiatives; however, more remains to be accomplished and a structured framework with specific goals is required.

Because it was envisioned this Workshop would build on past CIRS meetings on the topic of patient engagement, Scientific Director, CIRS, **Dr Neil McAuslane** presented highlights and recommendations from those Workshops from 2012-2014. In all the meetings, participants were clear that the voice of the patient should be heard throughout the lifecycle of medicines. Other themes emerged from Syndicate discussion at the Workshops:

- Surveys should be conducted to gather stakeholder input from patients regarding their involvement and expectations and from companies, regulators and HTA assessors to identify when where and how to best engage patients.
- We should build on work being done and learn from positive and negative experiences from HTA and regulatory agencies, IMI, other initiatives and sectors and from patient education and training initiatives such as EUPATI.
- There is a need for a growing body of guidances and publications on patient engagement, methodologies and data collection and on how to manage specific issues; eg, such as conflict of interest.
- We can benefit from research to review and expand on disease-specific drug development guidelines and to evaluate how best to incorporate patient input; to frame the questions to be asked of patients; and to determine the optimal structure for patient information to reflect benefits, risks and uncertainties.
- Transparency is needed to inform patients who have participated in trials about which treatments they received and the study outcomes.
- Utilisation of new technologies should be optimised to keep patient interactions simple and to employ tools such as smart phones and electronic case reports to collect real world data
- CIRS should continue to include patients, companies, regulators, health technology assessors, payers and healthcare professionals at its Workshops to encourage cross stakeholder learning.



A coordinated framework for patient interaction is needed

Patient engagement is now seen as a prerequisite in the development of new medicines. **Nicola Bedlington,** *Secretary General, European Patients Forum* explained that the Innovative Medicines Initiative (IMI), through the European Patient Academy on Therapeutic Innovation (EUPATI) and PATIENT SMART is contributing to a more structured, systematic approach to that engagement throughout the EU. Ideally, these programmes can be made permanent to realise their maximum impact. Furthermore, training and education are vital – more patient experts are needed and ongoing continuous patient education is key. Appropriate mechanisms to involve patients at the right time in the right way require a robust platform that evolves over time as a core resource for all stakeholders. Global initiatives such as Patient-Focused Medicines Development (PFMD), a joint patient industry initiative that aims to make medicines research and development more patient-centric, will buttress work in Europe and transfer it into other regions. Patients' engagement in medicines R&D is complemented by patient organisations' critical advocacy role at both the EU and national levels on health and social policy as well as on pharmaceutical policy per se. All of this must be set in the wider equity and access context.

Dr Anton Hoos, *Head of Medical, Europe, Amgen, Switzerland,* agreed that all stakeholders must work together to develop a joint framework for patient involvement in drug development and for regulatory and access decisions. Although historically stakeholders often acted in functional and geographic isolation leading to suboptimal outcomes and higher development cost, industry has recognised the need to change its processes for the development and life cycle management of medicines. Dr Hoos cited ongoing efforts to develop a coordinated framework for patient engagement such as Patient SMART, the IMI platform to sustain patient engagement in therapeutic innovation supported by European Patient Forum and the European Organisation for Rare Diseases and Accelerated Development of Appropriate Patient Therapies (ADAPT) SMART. ADAPT SMART is an enabling platform for the coordination of Medicines Adaptive Pathways Patients (MAPPs) activities, investigating tools and methodologies and enabling communication among all stakeholders.

Recognising the importance of patient engagement, regulatory and health technology assessment agencies are identifying and working to overcome challenges.

Dr Isabelle Moulon, *Head of Patients and Healthcare Professionals Department, Stakeholder and Communication Division, European Medicines Agency* explained that a revised framework for patient participation, consultation and information is in place at the EMA. Currently patients are involved in EMA decision making as members of EMA governance and scientific committees, the Management Board, Committee for Orphan Medicinal Products, Paediatric Committee, Committee for Advanced Therapies and Pharmacovigilance and Risk Assessment Committee. Although no patients are members of the Committee for Human Medicinal Products (CHMP) a pilot is ongoing for patient involvement in CHMP discussions and the feasibility of eliciting patients' values and preferences is being explored. Challenges to patient involvement include the issue of understanding and valuing patients' versus citizens' perspectives; using the most appropriate methodologies for patient involvement and whether they should vary based on their aim; the role of healthcare practitioners in contextualising patient input; the optimal use of available information including that from health registries and social media; and the identification and address of legal, regulatory and financial issues that could give rise to procedural barriers.

Dr Petra Dörr, *Head of Communication and Networking, Deputy Director, Swissmedic* reported that a recent re-alignment at the agency includes dialogue with patient and consumer organisations and the active provision of information targeted to patient/consumer needs via a dedicated entry point on the Swissmedic website, the use of appropriate communication tools and the involvement of patient/consumer organisation representatives in pre-defined work areas. A two-year pilot is ongoing for the establishment of a Swissmedic working group with patient and consumer organizations and this pilot will be evaluated after 2 years. As of September 2015, Swissmedic is involved with 15 patient organisations and two members are currently performing the first EUPATI training course. Dr Dörr stressed that regulator-patient cooperation has to be tailored to the specific situation and to the needs of the stakeholder group. In addition, time and financial constraints by patient and consumer organisations have to be taken into account and different level of knowledge about regulatory processes among working group members point to the extreme importance of training. Finally, time is needed for a change in mindset within established organisations.

Patient engagement has been important at the Swedish pharmaceutical and dental benefits agency Tandvårds- och Läkemedelsförmånsverket (TLV) since its origination in 2002. **Niklas Hedberg**, *Chief Pharmacist, TLV* reported that there are patient representatives on both the decision-making board and transparency committee and that TLV has interactions with patients in an increasing number of new drug applications. Furthermore, it is envisioned that as drug development changes over time, patient engagement will be increasingly important at the agency. A major challenges faced by all agencies is the identification and inclusion of the most relevant organisations. Strong organisations do not always represent patients with the greatest medical needs and vice versa. Other challenges comprise how to initiate the right discussion with the right person, and the need for agencies to be relevant and correct but not bureaucratic. Building trust among stakeholders takes time and stakeholders must understand the ultimate responsibilities of each participant involved in healthcare decisions.

Dr Brian O'Rourke, *President and Chief Executive Office, CADTH* explained that patients are engaged in all CADTH programmes. Public or patient members participate on the CADTH Board and on appraisal committees; patient input is also solicited and considered when developing scientific advice and conducting reviews and in the deliberation and recommendation processes as well as in ongoing evaluations. In the review of 30 drugs by CADTH Common Drug Review (CDR) between March 2013 and June 2014, patient groups identified 119 important outcomes for drug assessment. CADTH review protocols included 89 of these 119 outcomes (75%), clinical trials included 61 (50%) and CDEC recommendations and reasons for those recommendations included 67 (56%). Although Canadian patient groups are eager to meaningfully participate in HTA, challenges such as identifying what evidence to collect and how to collect and present it need to be overcome. There is a steep learning curve for some patient groups and many have limited resources to fund ongoing participation. Opportunities include those for ongoing dialogue, education and training and the potential to introduce new patient engagement methods in evaluation and to demonstrate patient input has an impact.

Patient-reported and patient-relevant outcomes, patient preferences and patient perspectives: Industry and health technology assessment perspectives regarding the main challenges to measuring/collecting and utilising the information

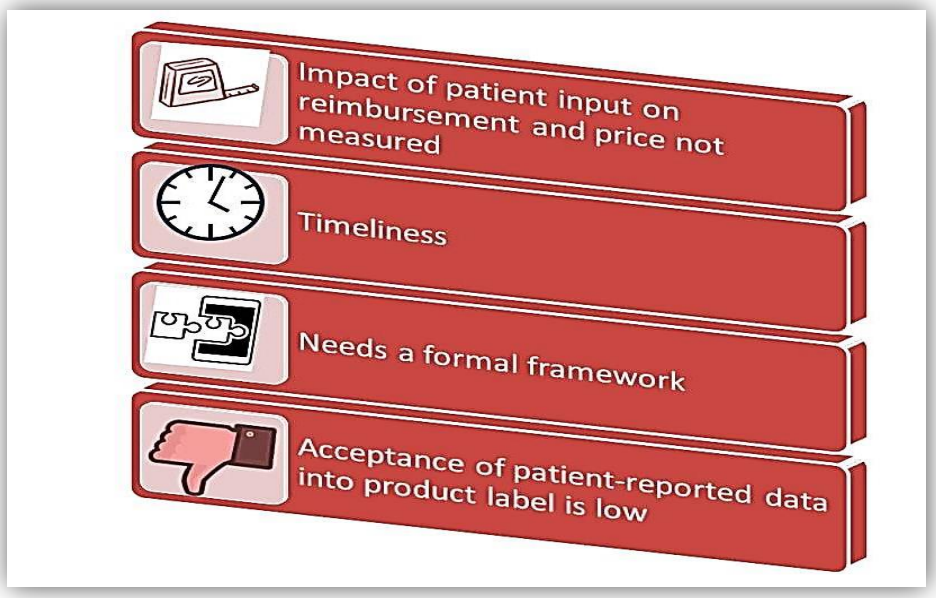
Measuring patient-reported outcomes or clinical outcomes assessments has been a core activity within industry for the past 15 years. However, as **Dr Indranil Bagchi**, Vice President and Head. Payer Insights and Access, Global Health and Value, Pfizer Inc, USA reported, these measurements have not been systematic, processes have been driven by clinician concepts of patient need rather than patient-derived needs and needs are not always incorporated early enough into the development process to have the greatest benefit for all stakeholders. Finally, the dissemination of PRO data into product labels is in it infancy.

There are excellent disease-specific PRO instruments, such as HAQ for rheumatoid arthritis and FACT for cancer and well-validated generic instruments such as EQ-5D and SF-36, but as we have gained precision in our estimates through the psychometric validation process, we have lost the power and value of the individual patient's voice. Our current challenge is to balance the qualitative patient voice, with the quantitative psychometrically validated instrument.

Pre-competitive collaborations are moving the science of patient input forward, with many EU collaborations through IMI1 and IMI2. HTAi work groups are addressing standard practices and evaluating outcomes of patient involvement and many US and global groups are driving collaboration. Ongoing and open dialogue among all stakeholders is necessary to address the many challenges. Involving patients not only in data collection, but also in identifying the right research questions and in determining the most feasible study design is critical. Systematic patient involvement in the drug development process will ensure products being developed that can and will be used.

The National Centre for Pharmacoeconomics (NCPE), Ireland provides the best, most informed summary of the evidence on the benefits, safety and value of medicines for the Irish healthcare payer within a formal health technology assessment framework. **Dr Roisin Adams,** *Deputy Head, NCPE* informed the Workshop that patient interaction with the NCPE consists of a combination of formal and informal input. Submissions from patient groups are invited during the HTA process and patients may also offer informal advice and guidance around the HTA process and recommendations, particularly in relation to assumptions or their perceptions of uncertainties. Patients interacting with NCPE receive training from EUPATI and the Irish Platform for Patient Organisations Science and Industry (IPPOSI). They are alos represented on some NCPE decision-making bodies such as their recent participation as part of the Hepatitis C treatment eligibility criteria review group.

Challenges include the consideration of patient-centric evidence within a formal framework, the management of conflicts of interest, the identification of contributors, timeliness of interactions, and the measurement of the impact of patient contributions on reimbursement and price. NCPE is currently examining how they might formally incorporate patient preferences via utility measurements into health technology assessments, how patient groups might work with physicians and HTA groups to gather outcomes and refine the methodologies to assess the balance of patient benefit-risk preferences for medicines, especially those with a smaller evidence base.

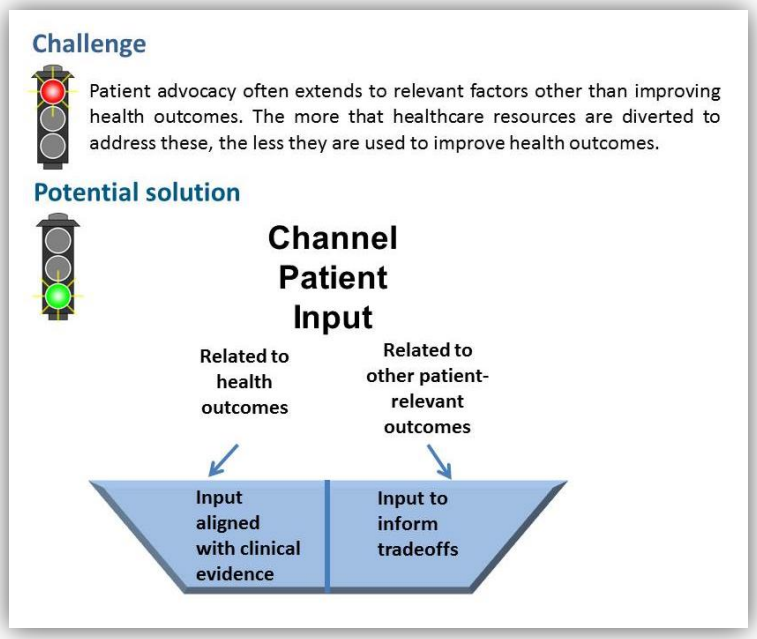


Hurdles to obtaining patient perspectives

Decision makers report on how various direct and indirect inputs from patients or citizens are used in assessments and the weight or influences these perspectives have on final decisions.

Dr Karen Facey, *Evidence Based Health Policy Consultant, UK* reported on the document *Values and Standards for Patient Involvement in HTA,* developed by the Health Technology Association International (HTAi) Interest Group on Patient and Citizen Involvement in HTA (PCIG). Use of the Nominal Group Technique at an international expert workshop elicited 44 potential principles that were refined and grouped into Values and Quality Standards by a steering committee using a Delphi survey to achieve consensus. *Values* were considered as desired goals and *quality standards* were specific actions to achieve those goals. Values include relevance, fairness, equity, legitimacy and capacity building. Quality standards for HTA assessments include proactive communication strategies; clear timelines; identified personnel for patient support; documentation of patients' perspectives and experiences and report of influence of patient contributions on conclusions and decisions and feedback and suggestions for future involvement.

Dr Simon Fifer, *Manager, Research Development, Institute for Choice***,** *University of South Australia* presented a case study of a discrete choice experiment that sought to elicit and quantify patient values in a systematic way for the purpose of treatment evaluation for chronic lymphocytic leukaemia (CLL). The qualitative data that were used to construct the quantitative survey on CLL were accrued from 12 in-depth face-to-face and telephone interviews regarding the social, physical, financial and emotional impact of CLL. An online survey of patients used that information to elicit CLL treatment preferences. The resulting dashboard of results could be used to conduct side-by-side comparisons of therapies for specific diseases, allowing industry to determine how well their products are aligned with patient preferences and for individual patients to make treatment decisions with their healthcare professionals.



Channelling patient advocacy

Dr Susan Morgan, *Medical Assessor, Medical and Healthcare Products Regulatory Agency (MHRA), UK* observed that patients are increasingly present in the review process at the agency. Examples of patient participation in MHRA include the Patient Group Consultative Forum piloted in 2013-2014, which consisted of 50 to 60 patient groups or individuals who acted as an Agency resource and took part in various projects. Another example, the Lay Forum, provides input to Expert Advisory Groups and the Commission on Human Medicines. Members must be skilled communicators, be able and prepared to contribute actively to the work, have the ability to understand and analyse often complex issues including technical data and use it to make decisions, be able to take an objective view, seeing issues from all perspectives and challenge constructively. Challenges to MHRA patient participation include determining how to engage the many rather than the few in a meaningful fashion, how to avoid the unintended consequences of these interactions, how to engage those without a voice and how to translate complex concepts in order to elicit meaningful interactions.

HTA systems actively seek inputs from patients to inform their appraisals and the output of HTA systems shows increasing influence of these contributions and of the broader perspectives of citizens on HTA decision making. **Andrew Mitchell**, *Strategic Adviser, Evaluation, Department of Health, Australia* pointed out, however, that patient participation raises important challenges for HTA systems and no ideal model has yet been established for bringing the public or the patient into the HTA decision-making domain. Identifying the truly representative patient viewpoints is difficult and by the time of HTA consideration, the true patient viewpoint is usually focussed on subsidised access to enable equity of access. In addition, patient advocacy often extends to relevant factors other than improving health outcomes and some elements of patient advocacy raise difficult questions of relevance. The dilemma is that the more healthcare resources that are diverted to address these issues, the less they will be used to directly improve health outcomes. A possible option to meet some of these challenges is to channel patient inputs into two streams, one, to inform HTA decision making and related only to health outcomes and aligned with available clinical evidence and the second, related to other patient-relevant consequences that can inform the broader trade-offs and any discussion about the limits of HTA.

Research has shown that there is public consensus regarding the importance of patient input in the development of medicines, and patients have taken a proactive role in this engagement.

Set up in 2001, The EU Health Policy Forum was developed to provide a mechanism to ensure that EU health policy was transparent and respondent to the needs of the public. **Sevala Malkic,** *Policy Officer, European Commission, Belgium* reported that the renewed Health Policy Forum will evolve from a consultative body into a multilateral communication mechanism which will be composed of three axes: an internet platform, providing online discussion and collaboration; regular meetings and a biennial Summit, providing targeted thematic discussions; and an annual Health Award for the best practice by non-government organisations in Member States. To foster patient empowerment, the European Commission also funds EMPATHiE, an ongoing pilot project on self-care in chronic disease. Finally, the Stakeholders Forum, the stakeholder advisory group of the European Network for Health Technology Assessment (EUnetHTA) was initiated to ensure transparent engagement with a broad range of stakeholders and comprises representatives from patients and healthcare consumer organisations, healthcare providers, payers and industry. Patient experience in the EUnetHTA Shaping European Early Dialogues (SEED) for health technologies Consortium, which is composed of 14 European HTA agencies, has been positive, with participants finding that the meetings were very open to patient views and featured productive dialogue.

Prof Dr Irina Cleemput, *Senior Health Economist, Belgian Health Care Knowledge Centre (KCE), Belgium* explained that a two-round Delphi survey was conducted among 187 participants investigating the possible models for public and patient involvement in health care decision-making processes; the survey probed the acceptability of these models to Belgian stakeholders**,** the perceived risks and benefits and the preferred model. The majority of respondents (72%) thought that patient or public involvement was important or very important. More than 65% of participants felt that timing and process, training and administrative and financial support were critical to maximise patient involvement.

This organisation also conducted a study of the feasibility and acceptability of models for citizen and patient involvement. The benefits of this involvement included the identification of needs and priorities; the building of expertise through experience; and the ability to move beyond the financial dimension of health technology assessment. Barriers to effective patient involvement included the potential of influence by other stakeholders; difficulty in expressing a broader societal opinion; lack of financial or logistical means of participation; and issues around understanding personal subjectivity. The majority of participants preferred involvement in the form of sharing information and consultation and agreed that actions to raise awareness are needed.

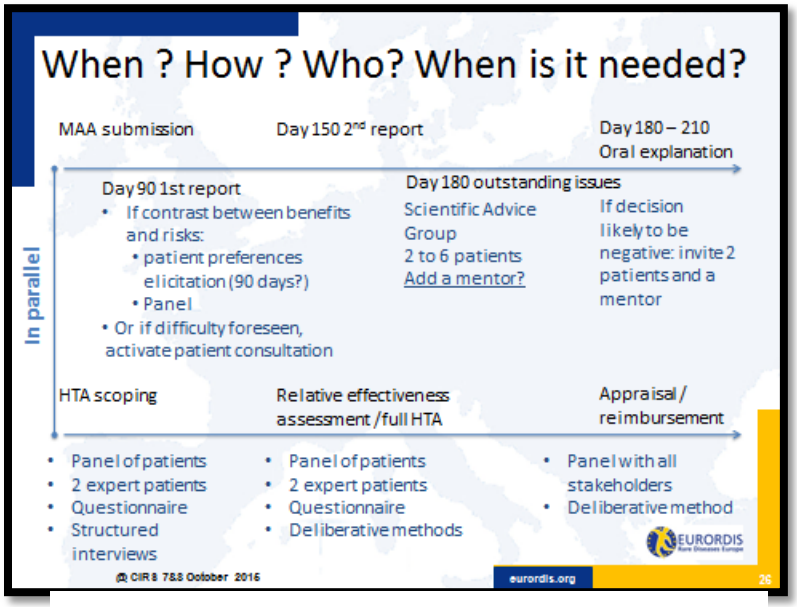
Patients may be involved in healthcare in a myriad of ways, such as taking part in a research study or a survey, by telling the public about what they are doing or planning and why, by listening and getting feedback, by actively collaborating in research, drug development or approval, by being a voting committee member, by identifying priorities or by performing their own research through interviewing writing or reviewing. **Dr Julian Walker,** *Director of Research and Development, Consultant Clinical Psychologist, Avon and Wiltshire Mental Health Partnership NHS Trust, UK* said that regardless of the method, patient involvement provides information that cannot be obtained in any other way, information that may counter what clinicians think of as obvious and information that improves focus and efficiency. This is consistent with observation reported elsewhere and consistent with the goals of involvement, which include efficiency, quality and satisfaction. Effective patient involvement improves research participation and compliance. Dr Walker offered recommendations to optimise patient involvement including building on existing experience to optimally engage patients, ensuring that any new work is evidence based, always using plain language when communicating with patients, providing extra efforts in terms of time, careful explanation, support and training, having clear, shared and realistic expectations, genuinely valuing people, keeping them informed and enjoying the interactive experience.

Making the best use of patient perspectives

Future perspectives – How different stakeholders see the patient engagement landscape and the role of the patient evolving to support their decision-making processes

Dr Isabelle Stöckert, *Vice President, Head Global Regulatory Affairs, EU CAN Pharma & EU Consumer Care, Bayer Pharma AG, Germany* proposed an optimistic view of 2020 that will include disruptive changes in drug therapy in which technological progress such as gene technology and immunotherapy will provide paradigm shifts from treatment to prevention and cure in some indications, The empowered patient will be central in the near future, as new technology will be at hand for patients and will be used in drug development and in communication of drug information between all stakeholders in the healthcare system. In addition there will be continuing pressure for value and real-world evidence and the desire for rapid access to innovative medicines will continuously mount. This requires an "all hands on deck" multi-stakeholder approach to drug development, approval and access. Finally, patient preference data and patient-reported outcomes will be integral part of development programs and of the dossiers provided to HTAs, payers and regulatory authorities to support the patient voice in decision making.

Although there are currently many activities to involve patients in healthcare decision making, Dr **Tomas Salmonson**, *Chair, CHMP, EMA* is convinced that there is much more to be accomplished. There are many reasons for regulators to encourage interaction with patients including their responsibility to relate with those they represent; their need to incorporate patient values during decision making and their knowledge that transparency and inclusion will enhance respect for the regulatory system. Patient input can also help regulators communicate their decisions to a broader audience and supports the rationale for those decisions. Patient input can be a valuable perspective when treatment guidelines need to be updated. Most importantly, regulators can use patient knowledge and experience to help patients reduce risks and make the best possible decisions about their own healthcare. Dr Salmonson suggested that stakeholders should make better use of technology to help develop a feedback loop in which patients can provide updates on the results of their care, regulators and health technology assessors can receive needed post-approval data to resolve uncertainties and clinicians can provide patients a comparison of their results with other similar patients or therapies.



A model for deliberative democracy for patient interaction.

At the National Institute for Health and Care Excellence (NICE), patient involvement is evolving through a focus on the big picture, using regular methods and processes that include all stakeholders through public consultations and guidances. According to **Heidi Livingstone,** *Senior Public Involvement Adviser, National Institute for Health and Care Excellence, UK,* current patient involvement includes the presence of two lay people on each appraisal committee and the participation in scope development, written submissions of evidence; the nomination of patient experts and the comment on draft documents by national patient groups. In addition, individual patients can attend as patient experts and provide personal statements and anyone can comment on draft recommendations. NICE will require even more patient input to respond to the adaptive licensing and accelerated access that is envisioned for the future and will be reviewing its methods and processes, including those for patient and carer involvement through such mechanisms as patient experience surveys, which are currently ongoing or the patient expert survey or patient organisation research, both of which are in development.

Providing the patient perspective of the future, **François Houÿez**, *Director, Treatment Information & Access, European Organisation for Rare Diseases (EURORDIS)* outlined the rationale for patient participation in healthcare decision making: to provide guidance on research priorities and relevant endpoints; to enrich regulatory outcomes by complementing it with the views of those directly affected by regulatory decisions; to help in interpreting trial results or checking data quality; to increase confidence and trust in regulatory and HTA process and to improve communication on decisions. Programmes such as EUPATI are working toward increasing the number of trained advocates and European authorities are testing different methods for patient inclusion. Mr Hoyez suggested a model of deliberative democracy for patient interaction in which patient panels and patient experts are among decision makers using structured interviews and deliberative methods to make regulatory and reimbursement decisions at defined points in the life cycle of a medicine.

WORKSHOP ATTENDEES

Regulatory agencies		
Dr Silvia Cammarata	Scientific Secretariat of the Director General	Agenzia Italiana del Farmaco, AIFA, Italy
Dr Petra Doerr	Head of Communication and Networking, Deputy Director	Swissmedic, Switzerland
Sevala Malkic	Policy Officer	European Commission, DG Sante, Belgium
Andrew Mitchell	Strategic Adviser, Evaluation	Department of Health, Australia
Zoe Molyneux	Policy Lead, Accelerated Access Review	Office of Life Sciences, UK
Dr Susan Morgan	Medical Assessor	Medicines and Healthcare products Regulatory Agency
Dr Isabelle Moulon	Head of Patients and Healthcare Professionals	European Medicines Agency
Laura Oliveira	Head of RRAA EU procedures. Department for Human Medicinal Products	Spanish Agency for Medicines and Health Care Products (AEMPS)
Dr Tomas Salmonson	Chair	CHMP, EMA
Dr Eyal Schwartzberg	Head of Pharmaceutical Division	
Health services providers		
Hannah Antoniades	Associate Director of Research and Development	Avon & Wiltshire Mental Health Partnership NHS Trust, UK
Rosmin Esmail	Director	Alberta Health Services, Canada
Dr Julian Walker	Director of Research and Development, Consultant Forensic Clinical Psychologist	Avon and Wiltshire Mental Health Partnership NHS Trust, UK
Health technology assessment agencies and economic consultancies		
Dr Roisín Adams	Deputy Head	National Centre for Pharmacoeconomics, Ireland
Dr Irina Cleemput	Senior Health Economist	Belgian Health Care Knowledge Centre (KCE)
Dr Karen Facey	Evidence Based Health Policy Consultant	UK
Dr Simon Fifer	Manager, Research Development	Institute for Choice, University of South Australia
Prof Jonathan Fox	Chair	Scottish Medicines Consortium, UK
Niklas Hedberg	Chief Pharmacist	TLV, Sweden
Anne Lee	Chief Pharmaceutical Adviser	Scottish Medicines Consortium, UK
Heidi Livingstone	Senior Public Involvement Adviser	NICE
Dr Brian O'Rourke	President and CEO	Canadian Agency for Drugs and Technologies in Health
Prof Robert Peterson	Executive Director, Drug Safety Effectiveness Network	Canadian Institute of Health Research
Barbara Sabourin	Director General, Therapeutic Products Directorate	Health Canada
Prof Zbigniew Szawarski	Professor	PolAHTA (Polish Agency for Health Technology Assessment)
Prof Adrian Towse	Director	Office of Health Economics, UK
Patient and citizen organisations		
Nicola Bedlington	Secretary General	European Patients Forum
Patricia Furlong	President	Parent Project Muscular Dystrophy, USA
François Houÿez	Director of Treatment Information & Access	European Organisation for Rare Diseases (EURORDIS)
John Marsh	Patient Representative	Cancer Research UK
Sarah Richard	Health Services Research Manager	Myeloma UK
Alastair Kent	Director	Genetic Alliance, UK
Natacha Bolanos	Director, Patients and Public Affairs	GEPAC/AEAL, Spain
Universities		
Mikkel Sachs	Industrial PhD Student	University of Copenhagen, Denmark
Prof Sam Salek	Professor of Pharmacoepidemiology	University of Hertfordshire, UK
Dr Pieter Stolk	Project Manager	Escher, TI Pharma & Utrecht University, The Netherlands
Pharmaceutical companies and consultancies		
Stephane Andre	Head of EU International Regulatory Affairs	Roche, Switzerland
Dr Indranil Bagchi	Vice President and Head, Payer Insights and Access, Global Health and Value	Pfizer Inc, USA
Neil Bertelsen	Global Advocacy Manager (pipeline)	Bayer Pharma AG, Germany
Andrea Beyer	Senior Expert Statistician	Actelion Pharmaceuticals Ltd, Switzerland
Dr Mondira Bhattacharya	Infectious Diseases Therapeutic Area Head, Pharmacovigilance and Patient Safety	AbbVie Inc, USA
Dr Alison Bond	Regional Policy Lead, Global Regulatory Policy and Intelligence	Janssen Pharmaceutical, UK
Francesca Caprari	Head of Payer Policy	Sanofi, Italy
Deven Chauhan	Senior Director, Value Evidence Leader	GlaxoSmithKline,UK
Mireille Collombat	Group Leader, DRA Project Management	Actelion Pharmaceuticals Ltd, Switzerland
Dr Caroline DeSurmont-Ruchaud	Chief of Staff – Global Regulatory Affairs	Sanofi, France
Sheila Dickinson	Senior Quantitative Safety Statistician	Novartis, Switzerland
Maud Dirken	EMEA TA Lead Immunology	Janssen Biologics, The Netherlands
Dr Mark Finch	Associate Director, Global Regulatory Affairs	Shire, UK

Pharmaceutical companies and consultancies (cont)

Anna Forsythe	Head, Global Value and Access Strategy	Eisai Inc, USA
Amanda Foster	Director	GSK, UK
Claire Fraenkel	Project Director, New Product Registration	Institut de Recherches Internationales Servier, France
Adrian Griffin	Vice President, Global HTA and Reimbursement Strategy	Johnson & Johnson, UK
Dr David Guez	R&D Special Projects Director	Institut de Recherches Internationales Servier, France
Angus Gunn	Global Head Early Portfolio, PRO Strategy & BD, Global Market Access and Pricing	UCB BioPharma, UK
Dr Oliver Günther	Evidence & Value Development Lead Immunology	Merck KGaA, Germany
Dr Sanjay Gupta	Executive Director & Head, Health Economics & Outcomes Research	Daiichi Sankyo Inc, USA
Dr Michael Happich	Director, HTA BioMeds Canada & Europe	Eli Lilly and Company, Germany
Dr Adam Heathfield	Senior Director, Global Health and Value Innovation Centre	Pfizer, UK
Dr Anton Hoos	Head of Medical Europe	Amgen Europe, Switzerland
Mark Hope	Head of Global Regulatory Affairs	UCB BioPharma, Belgium
Dr David Jefferys	Senior Vice President, Global Regulatory, Government Relations, Public Affairs and European Product Safety	Eisai, UK
Prof Trevor Jones	Chairman	Simbec-Orion Group, UK
Dr Tanja Keiper	Associate Director	Merck Serono, Germany
Dr Jan Kilhamn	Vice President, Clinical Development EU	Biogen, UK
Dr David King	Director, Regulatory Policy and Intelligence	Shire Pharmaceuticals, UK
Charlotte Kornbo	Senior Director, Quality	Biogen, Denmark
Dr Hilje Logtenberg–van der Grient	Owner LvdG Consultancy / Member Scientific Committee ELPA	LvdG consultancy / ELPA, The Netherlands
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Marie-Laure Prud'homme	Market Access Payer Policy	Sanofi, France
Fiona Reekie	Director, Regulatory Affairs	Biogen, UK
Dr Paul Robinson	Executive Director, Patient Perspective	Merck, Sharp & Dohme, UK
Merete Schmiegelow	Senior Director, Regulatory Policy, Honorary Industrial Ambassador in Regulatory Science at the University of Copenhagen	Novo Nordisk A/S, Denmark
Soren Skovlund	Director, Patient Research, Health Economics and Outcomes Research – Insulin, Global Market Access	Novo Nordisk A/S, Denmark
Pam Smith	Vice President, Europe and Emerging Markets	AstraZeneca, UK
Dr Isabelle Stoeckert	VP, Head GRA EU CAN Pharma & EU Consumer Care	Bayer Pharma AG, Germany
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Geraint Thomas		Amgen, UK
Natalie Tolli	Regulatory VP, Global Labelling Strategy, Ad/Promo Compliance & Operations, Regulatory Policy and Intelligence	AbbVie, USA
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Dr Guy Yeoman	Vice President, Patient Centricity	AstraZeneca, UK

Centre for Innovation in Regulatory Science

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Dr Neil McAuslane	Director
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Professor Stuart Walker	Founder
Tina Wang	Portfolio Manager, HTA Programme