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

7 – 8 October 2015

PROGRAMME

Windsor Heathrow Marriott, Slough, UK

CENTRE FOR INNOVATION IN REGULATORY SCIENCE
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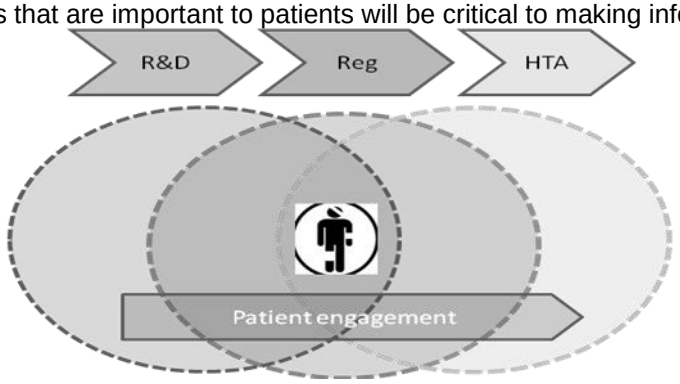
Centre for Innovation in Regulatory Science Workshop

Background

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

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 methodology 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?

The key questions for discussion are: What are the learning's 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?

This Workshop will build on CIRS Workshops on this topic conducted during 2012-2014 and will focus on the current process 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.

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

Venue

The Workshop will take place at the Windsor Heathrow Marriott Hotel, UK commencing at 09:00 on 7 October and finishing at 15:30 on 8 October 2015.

Style and Participation

Following the agreed practices for CIRS Workshops, the meeting will be for the benefit of CIRS members and allied organisations and the size will be limited to allow productive networking and discussions.

Day 1: 7 October 2015

08:30 Registration

SESSION 1: THE CURRENT PATIENT ENGAGEMENT LANDSCAPE: A LIFECYCLE APPROACH CHALLENGES AND OPPORTUNITIES	
09:00	**Chair's welcome and introduction** **Prof Trevor Jones,** Chairman, Simbec-Orion Group, UK
09:10	**Key outcomes and recommendations from previous CIRS Patient Workshops on:** *Assessment of benefits and harms and their relative importance for patients, industry and agencies* *The patient voice in clinical development: Can patients contribute to the benefit-risk assessment of new medicines?* *The patient's role in the benefit-risk assessment for the submission and review of new medicines* **Dr Neil McAuslane**, Director, CIRS
09:25	**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
09:50	**Integrated patient involvement in the lifecycle of a new medicine: Industry viewpoint** **Dr Anton Hoos,** Head of Medical Europe, Amgen, Switzerland
10:10	**Discussion**
10:20	**Break**
10:50 *11:10*	**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 Doerr,** Head of Communication and Networking, Deputy Director, Swissmedic
11:30	**How do patients view the approaches by regulatory agencies for engagement?** **Patricia Furlong,** President, Parent Project Muscular Dystrophy, USA
11:50	**Discussion**
12:00 *12:15*	**The HTA agency patient/citizen engagement landscape: Challenges and opportunities** **TLV approach – Niklas Hedberg,** Chief Pharmacist **CADTH approach – Dr Brian O'Rourke,** President and Chief Executive Officer
12:30	**How do patients view the various types of HTA engagement? Interactive panel discussion with patient representatives**
13:00	**Lunch**

Day 1: 7 October 2015

SESSION 2: MEANINGFUL PATIENT ENGAGEMENT: WHAT ARE BEST PRACTICES AND WHAT CAN BE MEASURED?	
14:00	**Introduction to Session 2**
14:05 *14.25*	**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 Helath and Value, Pfizer Inc, USA **HTA agency Viewpoint – Dr Roisin Adams,** Deputy Head, National Centre for Pharmacoeconomics**,** Ireland
14:45	**Discussion**
14:55 *15:15*	**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
15:35	***Discussion***
15:40	**Quality standards for patient/citizen involvement in HTA engagement - Dr Karen Facey,** Evidence Based Health Policy Consultant, UK
16:00	**Patient engagement – Industry case study – Dr Simon Fifer,** Manager, Research Development, Institute for Choice, University of South Australia
16:25	**Discussion**
SESSION 3: SYNDICATE DISCUSSIONS	
16:30	**Introduction to the syndicate sessions**
	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*
18:00	**Close of day one**
19:00	**Reception followed by workshop dinner**

Day 2: 8 October 2015

SESSION 3: SYNDICATE DISCUSSIONS	
08:30	**Continuation of syndicate discussions**
09:45	**Close of syndicate discussions and break**
SESSION 4: INTEGRATED PATIENT ENGAGEMENT FROM BENCH TO REIMBURSEMENT DECISION	
10:30	**Chair's Introduction - Prof Adrian Towse,** Director, Office of Health Economics
10:40	**Feedback from Syndicate Sessions and discussion**
	Patient engagement in the decision making process – How do other stakeholders perceive their role?
11:55	**Policy perspective - European commission – Sevala Malkic,** Policy Officer, European Commission, Belgium
12:10	**Society perspective** - **Prof Dr Irina Cleemput**, Senior Health Economist,, Belgian Health Care Knowledge Centre (KCE), Belgium
12:25	**Clinician perspective – Dr Julian Walker,** Director of Research and Development, Consultant Clinical Psychologist, Avon and Wiltshire Mental Health Partnership NHS Trust, UK
12:40	**Discussion**
12:45	**Lunch**
SESSION 5: ENABLING A FUTURE OF PATIENT-CENTERED HEALTHCARE AND SHARED DECISION MAKING: WHAT IS THE ROLE OF HTA, REGULATORY AGENCIES AND COMPANIES?	
13:45	**Chair's Introduction - Prof Adrian Towse,** Director, Office of Health Economics
	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?
13:50	**Industry vision** – **Dr Isabelle Stoeckert,** Vice President**,** Head Global Regulatory Affairs, EU CAN Pharma & EU Consumer Care, Bayer Pharma AG, Germany
14:10	**Regulatory vision – Dr Tomas Salmonson**, Chair, CHMP, EMA
14:30	**HTA vision** – **Meindert Boysen**, Programme Director Technology Appraisals, National Institute for Health and Care Excellence, UK
14:50	**Patients' vision – François Houÿez**, Director, Treatment Information & Access, European Organisation for Rare Diseases (EURORDIS)
15:10	**Discussion**
15:20	**Chair's closing remarks and close of workshop**