Commonality in Evidentiary Requirements across Regulatory and HTA Stakeholders

21 - 22 September 2016

PROGRAMME

Venue: Runnymede Hotel, Surrey, UK

CENTRE FOR INNOVATION IN REGULATORY SCIENCE
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V8 12/8/16

Centre for Innovation in Regulatory Science Workshop

Background

Commonality in evidentiary requirements across Regulatory and HTA Stakeholders

The current dynamics of bringing new medicines to market are being influenced by conflicts between the agendas of regulators, HTA and payers. Regulators are under pressure to develop methods to speed the approval process, including mechanisms such as flexible accelerated approval pathways, while maintaining an emphasis on safety, quality and efficacy. By contrast, there is an increasing pressure on payers to control spiralling healthcare costs via heath technology assessment of the clinical and cost-effectiveness aspects of new interventions. The two processes of licensing and reimbursement are introducing an additional uncertainty into drug development decisions as market approval does not necessarily mean that the product will be reimbursed through single payer formulary systems. While, historically the regulatory review and the consideration of products for formulary listing and reimbursement by healthcare providers *(health technology assessment* – HTA) have been entirely separate, the current dialogue around comparative effectiveness research may lead to a closer relationship between the two. The aim of this Workshop is to build on previous CIRS Workshops and recommendations that called for the development of more clarity around the *scientific basis for common evidentiary requirements* and encourage alignment of these needs across regulators and HTAs as such, this Workshop will focus specifically on these two stakeholders.

Over the last five years alignment of complimentary evidence generation for regulatory and HTA decision making has been seen as critical for effective and efficient development. Indeed recommendations from the March 2011 CIRS Workshop entitled "Evidentiary Requirements in Clinical Development: Synchronising phase III requirements to meet multiple needs", included: the undertaking of an HTA metrics and benchmarking survey; that HTA and regulatory authorities should seek agreement on the choice of endpoints and comparators; and the development of more clarity around scientific issues that can be aligned with increased early dialogue both between HTA agencies and HTA and regulators.

Since this Workshop, a number of initiatives involving scientific advice in early development have begun; these have included pilots for companies to receive joint stakeholder advice (HTA and Regulatory) to initiatives providing multi-stakeholder advice (multiple HTAs). In addition, companies themselves are benchmarking the activities they conduct during early development to identify how best to align the needs of the HTA in Europe and payer organisations in the US and licensing agencies to ensure both an effective and efficient development programme. This is being complimented by initiatives at the policy level within regions to ensure that duplication of effort is reduced and at the research level to understand the decision making processes and to identify if divergent decisions between regulatory and HTAs are due primarily to differences in the evidentiary requirements or due to other factors.

The aim of this workshop is to discuss the direction of change within the current landscape towards alignment and more synchronised decision making between agencies, how agencies are managing uncertainty by determining where the key differences are, and the implications of this to the changing development and approval models seeking increased flexibility in regulatory and access pathways.

Workshop Objectives

- **Discuss the progress made to align evidentiary requirements**, what the drivers have been and if this has improved efficiency and effectiveness of the development, approval and reimbursement process
- **Identify the areas where there are still major differences** that impede efficient and non-divergent decision making by regulators and HTAs
- **Make further concrete recommendations on how to ensure complementary evidence generation** and what is needed to mitigate the risk of mismatch of outcomes that can occur when a regulatory authority grants an approval that is not compatible with current HTA decision-making requirements

Venue

The Workshop will take place at The Runnymede Hotel, Surrey, UK commencing at 09:00 on 21st September and finishing at 16:00 on the 22nd September 2016.

Style and Participation

Following the agreed practices for CIRS Workshops, the meeting will be by invitation and the size will be limited to allow productive networking and discussions.

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Day 1: 21st September 2016

08:30 Registration

SESSION 1: MEETING THE EVIDENTIARY REQUIREMENTS FOR REGULATORY AND HTA(S) DURING NEW MEDICINES DEVELOPMENT: WHAT ARE THE KEY CONSIDERATIONS?	
09:00	**Welcome and opening remarks** **Lawrence Liberti,** Executive Director, CIRS
09:05	**Chair's welcome and introduction** **Prof Hubert Leufkens,** Chairman, Medicines Evaluation Board, Netherlands
09:15	**Has the gap between Regulatory and HTA evidence requirements narrowed?** *CIRS survey to identify changes since 2011 across stakeholders as well as stakeholders perception of the current landscape, key barriers and opportunities* **Tina Wang**, Manager, HTA Programme, CIRS
09:35	**Discussion**
09:45	**Getting to the right evidence during development to support both the registration and reimbursement decision: An achievable endpoint?** *Are their major evidentiary differences that companies need to consider? How could these be best managed? Are there still major barriers to Reg/HEOR collaboration within companies? Are certain types of products at greater risk for divergent Reg/HTA decisions because of the lack of evidentiary alignment?* **Regulatory agency viewpoint - Dr Tomas Salmonson,** Chair, CHMP, European Medicines Agency **HTA viewpoint – Niklas Hedberg,** Chief Pharmacist, TLV, Sweden **Industry viewpoint - Pam Smith,** Vice President –Europe & Emerging Markets Regulatory Affairs, AstraZeneca, UK
10:45	**Discussion**
11:00	**Break**
	Discussion: Potential Benefits of Early Advice and Dialogue
11:30	**Multiple HTA advice: Past learnings and future development of multiple HTA agency early dialogue within Europe – Is a single European advice achievable** **Wim Goettsch,** Director, EUnetHTA JA3 Directorate, Zorginstituut Nederland
11:45	**Joint HTA and Regulatory scientific advice — Is this helping align regulatory and HTA thinking in the development space?** **Rob Hemmings,** Head of Licensing Division's Statistics Unit, MHRA, UK
12:00	**Company Perspective: Are industry's needs best served by current dialogue models and, if not, what changes would be helpful?** **Francesca Caprari,** Head of Payer Intelligence and HTA, Sanofi SA, Italy
12:15	**What types of interactions beyond scientific advice between Regulatory and HTA agencies can be of use?** **Dr Brian O'Rourke,** President and CEO, Canadian Agency for Drugs and Technologies in Health
12:30	**Internal company alignment/dialogue (between regulatory and health outcomes) – Is this being achieved and does this enable better scientific advice requests?** **Christine Mayer-Nicolai,** Head Global Regulatory and Scientific Policy, Merck KGaA, Germany
12:45	**Discussion**
13:00	**Lunch**

Day 1: 21st September 2016

SESSION 2: FIT-FOR-PURPOSE LIFE-CYCLE MANAGEMENT - HOW TO BEST CO-ORDINATE THE NEEDS OF REGULATORS AND HTAS PRE- AND POST-LAUNCH	
14:00	**Chairman's Introduction** **Dr Sean Tunis,** Founder and CEO, Center for Medical Technology Policy, USA
14:05	**Ensuring there is a collective responsibility for the development of a high-quality evidence pool** *As drug development is based upon the serial development of evidence, how can we ensure that all stakeholders are part of the decision making process and what is the role of the regulator and the HTA in participating in the knowledge build-up that will foster the development of innovative medicines that will meet healthcare needs?* **IMI Adapt Smart – Prof Sarah Garner,** Associate Director, Science Policy and Research, National Institute of Health and Care Excellence (NICE) UK
14:30	**Discussion**
	Synchronisation of regulatory and HTA decision making – How do systems need to change to meet this goal? *How will novel products utilizing different flexible regulatory pathways challenge any reduction in the timing between the regulatory and HTA decision making? Will agencies need to adapt or work better together to enable the evidence submitted to meet their needs? How will companies efficiently manage their evidence generation to meet the synchronisation of regulatory and HTA decision making?*
14:40	**HTA viewpoint - Andrew Mitchell,** Strategic Adviser, Evaluation, Department of Health, Australia
15:00	**Regulatory viewpoint – Marion Law,** Director General, Therapeutic Products Directorate, Health Canada
15:20	**Company viewpoint – Adam Heathfield,** Senior Director, Global Health and Value Innovation Centre, Pfizer, UK
15:40	**Discussion**
15:55	**Introduction to syndicate sessions**
SESSION 3: SYNDICATE SESSIONS	
16:00	**Break and all delegates to syndicate rooms**
16:15	**Syndicate sessions** **Topic A: Early dialogue: How to use input from a variety of stakeholders to effectively ensure a development plan that best meets the needs of regulatory and HTA agencies?** **Chair: Dr Thomas Lonngren,** Independent Strategy Advisor, PharmaExec Consulting Filial SE, Sweden **Rapporteur: Louise Gill,** Regulatory Head – Europe and Canada, Global Regulatory Affairs, GlaxoSmithKline, UK **Topic B: Integrating regulatory and HTA evidence requirements into clinical programmes for standard and novel products – How can this best be achieved?** **Chair: Prof Jonathan Fox,** Chair, SMC **Rapporteur: Marci English,** Director HEOR, Astellas Pharma US Inc, USA **Topic C: Post-licensing evidence generation to support accelerated regulatory pathways and HTA decision making needs - How do we narrow the uncertainty gap?** **Chair: Dr Sandra Kweder,** Deputy Director, Europe Office, FDA, USA **Rapporteur: Claudine Sapede,** Global HTA and Payment Policy Lead, **F**. Hoffmann-La Roche Ltd, Switzerland
18:00	**End of Session**
19:00	**Drinks reception**
19:30	**Workshop dinner**

DAY 2: 22nd September 2016

SESSION 3: SYNDICATE SESSIONS	
08:30	**Syndicate sessions resume**
10:00	**Break**
SESSION 4: SYNDICATE FEEDBACK AND PRESENTATIONS	
10:45	**Chairman's Introduction** **Prof Sir Alastair Breckenridge**
10:50	**Feedback of syndicate discussion and participants viewpoint following each syndicate discussion**
11:50	**How are patients focusing their activities to use their voice to inform both the regulatory and HTA decision making?** **Overarching patient group activities - Nicola Bedlington,** Secretary General, European Patients Forum, Belgium **Disease specific patient activities** - **Melanoma** - **Dr Bettina Ryll,** Founder, Melanoma Patient Network Europe **Company perspective - Sonja Pumplün,** Head, Global Regulatory Affairs, Actelion, Switzerland
12:50	**Discussion**
13:00	**Lunch**
SESSION 5: BEYOND HTA AND REGULATORY COORDINATION	
14:00	**Chairman's Introduction**
14:05	**Panel Discussion: Enabling effective and efficient drug development: Where are companies, regulatory agencies, licensing authorities and HTA agencies going in the next 10 years and what is the pathway to the future? (10 minutes talks)** *What needs to be changed or considered from a strategic, technical and process view point to enable companies, licensing authorities, and HTA agencies to ensure patient access to innovative medicines?* **European Regulatory Agency Perspective – Prof Hans-Georg Eichler,** Senior Medical Officer, European Medicines Agency **European HTA Agency Perspective – Meindert Boysen,** Director, Technology Appraisals Programme, NICE, UK **Academic Perspective– Prof Adrian Towse,** Director, Office of Health Economics, UK **Company Perspective - Shane Kavanagh,** Vice President, Health Economics, Janssen NV, Belgium **Patient Perspective - Jean Mossman,** Senior Associate Director (Honorary), London School of Economics, UK
15:00	**Discussion**
15:45	**Closing remarks and end of workshop**