CMR International
INSTITUTE FOR REGULATORY SCIENCE

Workshop on

Review and Reimbursement:
Aligning the needs and requirements in clinical development

23 - 24 March 2010

Mandarin Oriental,
Washington DC, USA

INSTITUTE FOR REGULATORY SCIENCE
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Background

The current dynamics of bringing new medicines to market are being influenced by conflicts between the agendas of regulators and payers. Regulators are under pressure to develop methods to speed the approval process, including mechanisms such as accelerated approvals, while maintaining an emphasis on safety, quality and efficacy. By contrast, there is an increasing pressure on payers to control spiralling healthcare costs via the assessment of clinical and cost-effectiveness. The two processes of licensing and reimbursement are introducing an additional uncertainty into industry drug development decisions as market approval does not necessarily mean that the product will be reimbursed. 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.

This Workshop has been jointly developed by the Institute and the Office of Health Economics UK to specifically address the related issues of overlap and/or differences in requirements between licensing, health technology assessment, formulary listing and reimbursement, and additionally the potential for mismatch of outcomes, in the multi-payer healthcare system of the USA. The current Workshop follows the Institute's September 2009 workshop: *Review and Reimbursement: a special case for better cooperation* that focussed on the single-payer systems of Australia, Canada, Europe and Switzerland. At the September workshop, the implications of the current experiments into information sharing, alignment of technical requirements and even joint scientific advice between regulatory authorities and HTA agencies was explored.

This current workshop will look at

1) How these experiments in improving dialogue and efficiency may or may not be applied in the US context given the different mandates and requirements of Federal and private technology assessors and payers.
2) How the changing requirements of regulatory authorities in other nations as a result of incorporation of HTA / Payor requirements may impact on harmonisation of scientific advice given by the FDA and these nations and also on drug development programmes in general.
3) Mechanisms by which the potential mismatch of evidence requirements by the FDA and HTA/Reimbursement authorities might be mitigated.

General Objectives

- **To improve efficiency of global development:** by identifying areas of overlap between the evidence requirements of HTAs and the regulatory authorities in drug assessment and to discuss mechanisms by which dialogue and information sharing may minimise duplication.
- **Rationalisation of outcomes:** to identify mechanisms 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 requirements.
- **To develop a white paper:** the recommendations of the two Workshops and the supporting survey will be used to create a white paper that addresses the global implications of any changes to the current model of keeping scientific review separate from health technology assessment by aligning the evidence needs and requirements for licensure with those of the evaluator in order to achieve greater efficiency of process and predictability of outcome.

Day 1: Tuesday 23 March 2010

08.30 **Registration**

Session 1: Review and Reimbursement – Understanding the Dynamics and How They are Evolving		
09.00	**Chairman's welcome and introduction: the changing face of healthcare in the USA**	**Dr Garry Neil,** *Corporate Vice President, Johnson & Johnson, USA*
Healthcare policy reform in the USA and the global implications for the development of new medicines:		
09.10	**FDA perspective**	**Dr Janet Woodcock**, *Director, CDER, Food and Drug Administration, USA*
09.30	**Health Insurer perspective**	**Dr Murray Ross**, *Vice President, Kaiser Foundation Health Plan; Director Kaiser Permanente Institute for Health Policy, USA*
09.50	**Discussion**	
10.00	**Break**	
Enabling effective and efficient drug development: Where are licensing authorities, HTA agencies and decision makers going in the next 10 years and what is the pathway to the future?		
10.30	**Industry perspective**	**Dr Freda Lewis-Hall**, *Senior Vice President, Chief Medical Officer, Pfizer Inc, USA*
10.50	**FDA perspective**	**Dr Janet Woodcock**, *Director, CDER, Food and Drug Administration, USA*
11.10	**HTA / Decision Maker perspective**	**Dr Clifford Goodman**, *Vice President, The Lewin Group; Chair, MEDCAC, USA*
11.30	**Discussion**	
Bridging the gap between the requirements for registration and the requirements of health outcomes for CER and reimbursement decisions		
11.45	**FDA perspective**	**Dr Robert Temple**, *Deputy Center Director for Clinical Science, FDA, USA*
12.05	**HTA perspective**	**Dr Naomi Aronson,** *Executive Director, Blue Cross/Blue Shield Association, USA*
12.25	**Industry perspective**	**Dr Tim Garnett,** *Senior Vice President and Chief Medical Officer, Eli Lilly & Company, USA*
12.45	**Discussion**	
13.00	**Lunch**	

Session 2: Syndicate sessions

14.20	**Introduction to the Syndicate Sessions**	**Dr Franz Pichler,** *Manager, HTA Programmes, CMR International Institute for Regulatory Science*
14.40	**Syndicate Discussion Groups**	
	Aligning the needs and requirements in clinical development *The American Recovery and Reinvestment Act 2009 has provided a stimulus supporting increased comparative effectiveness research in part in order to help with reducing the increasing rate of health care expenditure. How will CER impact on the choices of which drugs to take forward in development programmes? Will CER promote greater risk taking by moving development towards more innovative medicines or, alternatively, will this change the post-authorisation marketing strategies of sponsors via risk-sharing or the targeting of high-responder sub-populations? What might the global drug development implications be once CER becomes implemented in the US?*	Chair: **Dr Peter Doukas**, *Dean and Professor, Temple University School of Pharmacy, USA* Rapporteur: **Dr Linda Harpole**, *Vice President, Global Health Outcomes, GlaxoSmithKline, USA*
	Getting to the right evidence for registration and reimbursement: An achievable endpoint? *What would be required for agreement prior to the drug development process on the health priorities for new medicines in order to meet registration and HTA criteria for approval and reimbursement? What are the technical requirements within a drug development plan that can be aligned to suit both sets of requirements? Can the FDA and CER bodies reach consensus on biomarker choice, endpoints, and the development of comparator-arms of trials.*	Chair: **Dr Brian O'Rourke**, *Acting President and CEO, Canadian Agency for Drugs and Technologies in Health (CADTH)* Rapporteur: **Dr Zeba Khan**, *Vice President, Pricing and Market Access, Celgene, USA*
	How can patients have rapid access to new medicines where there is an unmet medical need? ***Accelerated approvals, conditional reimbursement & risk sharing*.** *Can we avoid a mismatch of outcomes between marketing approval based on surrogate endpoints and an HTA refusing to approve the same product due to lack of outcomes evidence? Should the post-marketing conditions from the regulator be linked with conditional reimbursement? Does risk sharing help? If in the future, there is an increase in the amount of early release of new innovative medicines (after Phase II) how will HTA and Payers react?*	Chair: **Prof. Hans-Georg Eichler,** *Senior Medical Officer, European Medicines Agency* Rapporteur: **Dr Marc Berger**, *Vice President, Global Health Outcomes, Eli Lilly & Company, USA*
	How can you use HTAs and Regulation to enable innovation? *The increase in requirements for both registration and reimbursement are increasing the costs of drug development and also shortening the effective patent time for new drugs with the knock on effect of potentially decreasing the number of new drugs being brought to market. What mechanisms can the authorities use in order to help stimulate innovation? Will harmonisation or alignment of the scientific and technical requirements for phase III clinical development or for post-marketing studies help achieve this goal or is more needed? Are new approaches to drug development necessary such as adaptive designs and early (end of phase II) release needed and if so, then how would the different stakeholders view such changes?*	Chair: **Prof Tomas Salmonson**, *Vice Chair, CHMP, EMEA, Medical Products Agency (MPA), Sweden* Rapporteur: **Dr Brian Seal** *Sanofi-Aventis*
15.45	Refreshments to be served to discussion groups	
17.45	**End of Syndicate Discussions**	
19.00	**Reception**	
19.30	**Dinner**	

Day 2: Wednesday, 24 March 2010

Session 3: Review and Reimbursement: Syndicate feedback and Global Implications of an Increasing Emphasis on HTA Requirements in Drug Development		
08.30	**Chairman's Introduction**	**Dr Paul Huckle**,*Senior Vice President, Global Regulatory Affairs, GlaxoSmithKline, USA*
Syndicate feedback		
08:35	**Rapporteur feedback of day one syndicate discussion**	
09.35	**Panel Discussion & General Discussion**	**Jean Slutsky**, *Director, Center for Outcomes and Evidence, Agency for Healthcare Research and Quality, USA* **Dr Sharon Levine,** *Associate Executive Director, The Permanente Medical Group, Kaiser Permanente, USA* **Dr Supriya Sharma,** *Director General, Therapeutic Products Directorate (TPD), Health Canada* **Dr John Jenkins***, *Director, Office of New Drugs, CDER, FD, USA*
10.40	*Break*	
Global implications of an increasing emphasis on HTA requirements in drug development		
11.00	**How the European Medicines Agency is adapting to include the needs of HTAs and payers?**	**Prof. Hans-Georg Eichler,** *Senior Medical Officer, European Medicines Agency*
11.20	**Alignment of HTA agency requirements in Europe and collaboration with the European Medicines Agency**	**Prof. Finn Børlum Kristensen,** *Director, Chairman of the Executive Committee, EUnetHTA Secretariat, National Board of Health, Denmark*
11.40	**Discussion**	
What is the pathway to the future of sustainable drug development?		
11.55	**What stepping stones should be put in place to shape the evolving regulatory landscape in the US to help meet the needs of all stakeholders while encouraging innovative drug development?**	**Dr Garry Neil**, *Corporate Vice President, Johnson & Johnson, USA*
12.25	**Discussion**	
12.40	**Lunch**	

Focus Session : Getting to the Right Evidence at Launch and Post-Launch		
13.40	**Chairman's Introduction**	**Prof Adrian Towse,** *Director, Office of Health Economics , UK*
13.45	**The challenge of the lack of evidence for HTA at launch: some solutions create more problems than they solve**	**Andrew Mitchell**, *Strategic Advisor Evaluation, Department of Health and Ageing, Australia*
14.05	**Coverage with evidence development**	**Dr Steve Phurrough**, *Chief Operating Officer & Senior Clinical Director, Center for Medical Technology Policy (CMTP), USA*
14.25	**Why is risk sharing largely a non-US phenomena?**	**Prof. Lou Garrison,** *Professor and Associate Director, Pharmaceutical Outcomes Research and Policy Program, University of Washington, USA*
14.45	**Pay for performance and risk sharing: an industry perspective**	**Alison Lawton**, *Senior Vice President, Global Market Access, Genzyme Corporation, USA*
15.05	**Discussion**	
15.25	**Chairman's Summary and Close of session**	
15.45	**Workshop ends**	