Workshop on

COMPANION DIAGNOSTICS

Challenges for developing, regulating and coverage decision-making at the beginning of the era of personalised medicine

18 - 19 September 2012
Sofitel Lafayette Square, Washington DC, USA

CIRS Centre for Innovation in Regulatory Science

Workshop Prepared By

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THE WORKSHOP

Convergence of biomedical technology is one of the keys to improving not only the safety, efficacy and effectiveness of drug treatments but in providing a new attitude to how disease is tackled with new approaches to prevention, screening, diagnosis, therapy, monitoring and disease management. It has been suggested that with improved knowledge of complex biological systems along with convergence of new technologies that this will lead to a 'P4' medicine model: predictive, personalised, preventative and participatory.

The advent of advanced genomic, proteomic and metabolomic technologies are enabling diseases to be interrogated with increasing sophistication leading to sub-setting of both diseases and patient populations. As the drug development industry moves from a 'blockbuster' to a 'niche-buster' model in response to the rising challenge of finding drugs of ever increasing effectiveness against general patient populations that are also safe and affordable, it would appear logical to develop companion diagnostics. But are companies currently incentivised to develop companion-diagnostics alongside their new therapeutics in order to define smaller target populations? It would also seem to be in the interests of regulators, coverage bodies and clinicians to support the development of companion diagnostics in order to meet their needs. Diagnostics should help in patient selection, reduce safety risks and aid in value-based pricing. Despite their promise, in practice there are a limited number of companion diagnostics in use. However, many companies are announcing a focus on developing companion genomic diagnostics for patient sub-grouping thus heralding an upcoming wave of companion diagnostic therapies in the near future.

In order for a shift to occur in the development and utilisation of companion diagnostics in both the development of new therapies and also in the clinical setting there needs to be clarity in the way these will be regulated and how they will need to demonstrate value in the HTA setting

OBJECTIVES

- To identify both the opportunities and the barriers to integration of companion diagnostic evaluations into patient care decision-making by looking at key issues surrounding product development, regulation, reimbursement, and clinical uptake.

- To discuss the role the different scenarios for utilisation of companion diagnostics have for patient access to new medicines of agreed high medical and economic value.

- To make recommendations on appropriate pathways for enhancing the opportunities and overcoming barriers to facilitate the appropriate and effective use of companion diagnostics in patient care decision-making.

VENUE

The Workshop will take place at the Sofitel Lafayette Square, Washington DC, commencing at 09.00 on Tuesday, 18 September and finishing at 13:00 on Wednesday, 19 September 2012

STYLE AND PARTICIPATION

Following the agreed practices for CIRS Workshops, the meeting participation is by invitation to maintain a size that encourages a neutral environment that promotes productive dialogue and networking. We aim to advance the debate and discussion around the subject of the workshop and to produce constructive recommendations based on the workshop activities.

Please contact Gill Hepton at ghepton@cirsci.org for further information.

Day 1:	Tuesday 18 September 2012
08:30	Registration
09:00	**Welcome** **Larry Liberti,** Executive Director, CIRS
09:10	**Chairman's Introduction to Workshop** **Dr Peter Honig**, Head, Global Regulatory Affairs and Patient Safety, AstraZeneca, USA
Session 1: Setting the scene	
09:15	**Putting the puzzle together: how do companion diagnostics fit into the whole health care system landscape** *An overview to describe current and future high-level opportunities and challenges for all the key stakeholders from product developers, regulatory reviewers, payers, and physicians to the discovery, validation and practical use – both in wide scale screening and in individual patients - of companion diagnostics. Where have they worked and where have they failed to date?* **Dr Ansgar Hebborn,** Head – Global Market Access, F. Hoffmann-La Roche Ltd, Switzerland
Session 2: Barriers and opportunities in drug-device combinations	
	Innovator perspective *What are the barriers to developing drug-diagnostics (practical, regulatory, etc). What does a developer look for when bringing such a product forwards? What are the implications for co-development of a drug-diagnostic when two companies are involved?*
09:40	**Drug Developer perspective** **Dr Greg Rossi**, Vice President, Payer and Real World Evidence, AstraZeneca, UK
	Regulator perspectives *What are the potential benefits of using diagnostics in disease sub-setting and in patient risk sub-setting from a regulatory perspective and what are the requirements that regulators would like to see? What are the challenges for regulators in reviewing drug-device combinations?*
10:00	**FDA perspective** **Dr Elisabeth Mansfield**, Director, Personalized Medicine Staff, Office of In Vitro Diagnostic Devices, Center for Devices, Food and Drug Administration DA, USA
10:20	**Health Canada perspective** **Kimby Barton**, Director of the Bureau of Cardiology, Allergy and Neurological Sciences, Health Canada
10:40	**Discussion**
10:50	**Break (30 min)**

	HTA perspectives *What are the evidentiary requirements that would benefit the evaluation of new combination products? What are the key aspects of trial design where both the drug and the diagnostic are novel?*
11:20	**Technology assessment needs for the USA** **Dr Naomi Aronson**, Executive Director, Technology Evaluation Center, Blue Cross and Blue Shield Association, USA
11:40	**Jean Slutsky,** Director, Center for Outcomes and Evidence, Agency for Healthcare Research and Quality, Dept of Health and Human Services, USA
	Coverage decision perspective *What are the potential benefits of using diagnostics in disease sub-setting and in patient risk sub-setting from a reimbursement perspective? What are the implications for value-based pricing of drug-device combinations? Are reimbursement requirements helping to drive the move to companion diagnostics?*
11:55	**US coverage decision maker perspective** **Brian Kelly,** Group President, Product Value Strategy, OptumInsight, United Health Care Group, USA
12:15	**Legal perspective** **Dr Bruce Quinn**, Senior Health Policy Specialist, Foley Hoag LLP, USA
12:35	**Discussion**
12:45	**Lunch (60 min)**

Session 3: Practical considerations for developing companion diagnostics	
13:45	**Stakeholder-informed methods for evaluating clinical utility of molecular diagnostics** **Dr Patricia Deverka,** Senior Research Director, Centre for Medical Technology Policy (CMTP), USA
14:05	**Discussion**
Session 4: Syndicate Discussions	
14:10	**Introduction to the Syndicate Discussions** **Dr Neil McAuslane,** Scientific Director, CIRS
Syndicate 1	**What strategies should companies employ for the effective development of drug-diagnostic combinations?** Chair: **Dr Tim Garnett**, Chief Medical Officer and Senior Vice President, Eli Lilly and Company, USA Rapporteur: **Dr Anant Murthy,** Executive Director, Global Market Access and Pricing, Celgene Corporation, USA
Syndicate 2	**Can regulatory and coverage clinical requirements, in particular, trial design and validation, be aligned?** Chair: **Dr Brian O'Rourke**, President and Chief Executive Officer, Canadian Agency for Drugs and Technologies in Health, Canada Rapporteur: **Todd Williamson**, Head of HEOR-US, Bayer, USA
Syndicate 3	**Companion diagnostics are challenging existing review processes. Are the regulatory and reimbursement processes for assessment fit for purpose? How can these be improved?** *What would be the 'ideal' changes needed to facilitate the review process? Are dossiers that are now being developed for companion diagnostics as well-designed as possible? What are the elements that can be improved to facilitate a timely, transparent value assessment of a companion diagnostic?* Chair: **Prof Hans-Georg Eichler,** Senior Medical Officer, European Medicines Agency Rapporteur: **Dr Rizwana Sproule,** Executive Director, Therapeutic Area Head, Global Regulatory Affairs and Safety, Amgen Inc, USA
17:30	**End of Syndicate Discussions and end of Day One**
19:00	**Drinks reception & dinner**

Day 2:	Wednesday 19 September 2012
08:30	**Chairman's Introduction** **Prof Sir Alasdair Breckenridge,** Chairman, Medicines and Healthcare products Regulatory Agency (MHRA), UK
Session 5: Horizon scanning: as we move into a world of personalised medicine will companion diagnostics become the norm?	
08:40	**What is the pharmacoeconomics model for companion diagnostics?** *An overview on the economics both from the view of the developers (pharma and diagnostic) and from the public policy perspective: What is motivating companies to develop companion diagnostics and what is the utility for coverage decision making? How does one determine the individual and community economic value companion diagnostics bring to the patient care/drug treatment scenario?* **Prof Lou Garrison,** Professor and Associate Director, Pharmaceutical Outcomes Research and Policy Program, University of Washington, USA
09:05	**Issues in post-marketing and clinical practice** *How do post marketing requirements impact on drug-diagnostics? Is this an area that would promote an HTA-Regulatory framework to enhance the use of diagnostics for patient selection, monitoring and the evaluation of benefit risk over the drugs lifecycle? What happens if a new diagnostic is invented that applies to a drug that specifies a different diagnostic in its market authorisation label?* **Prof Hans-Georg Eichler,** Senior Medical Officer, European Medicines Agency
09:30	**Who will pay?** *What are the reimbursement implications for both drug developers and coverage bodies in the case of new and expensive combination drug diagnostics that target population sub-sets? As many payers are defining reimbursement classes for new drugs (e.g. ASMR), then should the value lie with the drug or the diagnostic, and what happens when these are developed by different companies? What are the implications for reimbursement and fair competition where the patent life of the drug and diagnostic differ?* **Prof Adrian Towse**, Director, Office of Health Economics, UK
09:55	**General discussion**
10:00	**Break (45 min – to allow for check-out)**

Session 6: Feedback from Syndicates	
10:45	**Syndicate One** **Dr Anant Murthy,** Executive Director, Global Market Access and Pricing, Celgene Corporation, USA
11:00	**Syndicate Two** **Todd Williamson**, Head of HEOR-US, Bayer, USA
11:15	**Syndicate Three** **Dr Rizwana Sproule,** Executive Director, Therapeutic Area Head, Global Regulatory Affairs and Safety, Amgen Inc, USA
11:30	**Panel Discussion** **Dr Lucinda Orsini**, Group Director, GHEOR Oncology and Pharmacodiagnostics, Bristol-Myers Squibb, USA **Kimby Barton**, Director of the Bureau of Cardiology, Allergy and Neurological Sciences, Health Canada **Dr Naomi Aronson**, Executive Director, Technology Evaluation Center, Blue Cross and Blue Shield Association, USA
12:45	**Chairman's summary**
12:55	**Final word and close of workshop** **Larry Liberti**, Executive Director, CIRS
13:00	**Close of workshop**