Workshop on

EVIDENTIARY REQUIREMENTS IN CLINICAL DEVELOPMENT

Synchronising phase III requirements to meet multiple needs

31 March – 1 April 2011
InterContinental Hotel, Geneva, Switzerland

CIRS Centre for Innovation in Regulatory Science

Workshop Prepared By

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THE WORKSHOP

A drug development programme is by its very nature serial in building up knowledge about the new product. Therefore the evidence generation to decide about a new medicine's attributes requires the companies to build upon preceding studies. However companies today are increasingly obliged to consider more than the traditional requirements of quality, efficacy and safety as success is now measured by achieving both regulatory approval and reimbursement. In the current environment the regulator and the payer, who make separate decisions, review new medicines at essentially a similar time point and therefore often with the same underlying data. As companies plan future development programmes the question is how can inclusion of HTA requirements be conducted without making the drug development programme overly burdensome or complex?

The key area for debate is Phase III and particularly how practical changes to drug development design might improve efficiency, reduce duplication and mitigate against a potential mismatch of outcome of regulatory and payer approval in terms of the clinical evidence. There are several experiments (pilot projects) in regards to parallel advice from both the regulatory and the payer on the clinical development of a new product. The participants at this workshop will consider whether such advice is leading to better decision making and by discussing the different pilots, and will seek to identify what aspects of parallel advice programmes are most effective.

The foundations of this Workshop are based upon the discussions and recommendations from earlier meetings held by the Centre from 2007-2010 on the subject of regulation and reimbursement. This Workshop is intended for participants from regulatory, HTA and payer agencies and from the regulatory, health economics outcomes research, market access and clinical development functions from industry.

OBJECTIVES

- **Is there a collective responsibility to development of a high-quality evidence pool?** As drug development is based upon the serial development of evidence, what is the role of the regulator and the HTA in participating in the knowledge build up and how can industry avoid the temptation to overcomplicate evidence development to try to meet conflicting requirements?
- **Which phase III requirements can be aligned?** The key technical areas within drug development programmes where the differing requirements of the regulator and the payer might be aligned will be discussed
- **What is the best approach to achieving this alignment?** Discussion will focus on how such alignment might occur in practice, considering whether this would best occur on a drug-by-drug basis, by therapy area, or in forums
- **Does shared scientific advice help?** As payer agencies are now beginning to offer advice on development programmes, the Workshop will look at the current experience of pilot joint or parallel scientific advice and will seek to identify which approaches to advice works best for all parties

VENUE

The Workshop will take place at the InterContinental Geneva, Switzerland, commencing at 09.00 on Thursday, 31 March and finishing on Friday, 1 April 2011.

STYLE AND PARTICIPATION

Following the agreed practices for Institute Workshops, the meeting will be closed and the size will be limited to allow productive networking and discussions. Please contact Gill Hepton at ghepton@cmr.org for further information and a registration form.

Day 1:	Thursday 31 March 2011
08:30	Registration
09:00	**Welcome** **Larry Liberti,** Executive Director, Centre for Innovation in Regulatory Science
09:05	**Chair's Introduction to Workshop** **Dr Marcus Müllner,** Head of Agency, Austria Medicines and Medical Devices Agency (AGES PharmMed)
Session 1: Why is it important to seek ways to align the requirements of both the regulator and the payer in Phase III clinical development?	
09.10	**The business case for increased alignment of evidentiary requirements in Phase III: managing uncertainty and balancing risk with return.** **Dr Murray Stewart,** Senior Vice President, Head of Metabolic Pathways and Cardiovascular Therapy Area Unit, GlaxoSmithKline
09.50	**Why should the regulator and payer wish to align requirements, what's in it for them?** **Prof Adrian Towse,** Director, Office of Health Economics (OHE), UK
10:30	Break (30 min)
Session 2: Update on shared activities and accommodating requirements of multiple agencies in drug development programmes	
11:00	**The Australian pilot (TGA & PBAC) advice programme** **Andrew Mitchell**, Strategic Adviser, Evaluation, Department of Health and Ageing (DoHA), Australia
11:20	**The Swedish Presidency pilot (MPA & TLV) advice programme** **Bengt Ljungberg**, Scientific Director, Pharmacotherapy, Medical Products Agency (MPA), Sweden
11:40	**The EMA (EMA & multiple payer) Shared advice Pilot programme** **Prof Bruno Flamion**, Chairman, Belgian Committee for Reimbursement of Medicines (CTG/CRM), Belgian National Institute for Health and Disability Insurance (INAMI-RIZIV)
12:00	**Panel Discussion:** • **Dr Rohan Hammett**, National Manager, Therapeutic Goods Administration (TGA), Australia • **Niklas Hedberg**, Head of Department, The Dental and Pharmaceutical Benefits Agency (TLV), Sweden • **Adrian Griffin** Vice President, HTA and International Policy, Johnson & Johnson • **Seren Phillips,** Associate Director, Scientific Advice Programme, National Institute for Health and Clinical Excellence (NICE), UK • **Dr Nicola Course** Vice President, Global Regulatory Affairs, Europe, GlaxoSmithKline
13:00	Lunch (60 min)

Session 3: Key barriers to the inclusion of HTA requirements into Phase III: Comparators and Endpoints: ***Is it feasible to expect comparators and endpoints to meet the needs of both the Regulator and the HTA?***	
14:00	**Dr Greg Rossi**, Vice President, Global Health Economics and Pricing, Genentech
14:20	**Dr Mira Pavlovic,** Deputy Director, Health Technology Assessment Division, Haute Autorité de Santé, (HAS), France
14:40	Discussion
Session 4: Syndicate Discussions	
15:00	**Introduction to the Syndicate Discussions** **Dr Franz Pichler**, Centre for Innovation in Regulatory Science
15:15	**Recommendations of the 2011 HTAi Policy Forum** **Dr Clifford Goodman**, Senior Vice President, Lewin Group, USA
Syndicate 1	**How can companies best accommodate both regulatory and HTA requirements into their Phase III clinical programmes? What are the challenges and what approaches are most easily incorporated?** **Chair:** **Dr Patrick Keohane,** Vice President, Payer Evidence, AstraZeneca **Rapporteur:** **Dr Marie-Christine Minjoulat-Rey**, Head, Governance, Global Evidence and Value Development (EVD), sanofi aventis R&D
Syndicate 2	**How do we deal with areas of complexity in terms of differing regulatory and HTA needs, focussing on comparators and endpoints?** **Chair:** **Prof Bengt Jonsson,** Professor of Health Economics, Stockholm School of Economics (SSE), Sweden **Rapporteur:** **Dr Isaac Odeyemi**, Senior Director and Head of Health Economics and Outcomes Research, Astellas Pharma Europe
Syndicate 3	**What kind of scientific advice model is most effective?** **Chair:** **Dr Petra Dörr**, Head of Management Services and Networking, Swissmedic **Rapporteur:** **Dr Mel Walker**, Senior Director, Value Expert Engagement & Collaborations, GlaxoSmithKline
18:30	End of Syndicate Discussions and End of Day One
19:00	Reception
19:30	Dinner

Day 2:	Friday 1 April 2011
08:30	**Chair's Introduction** **Prof Sir Alasdair Breckenridge,** Chairman, Medicines and Healthcare products Regulatory Agency (MHRA), UK
Session 5: Working towards greater clarity of roles	
09:00	**What is industry looking for from Regulatory, HTA, Payer agencies, how should roles be defined and where could interaction between these agencies be of use?** **Dr Pierre Sagnier,** Vice President, Global Market Access, Development Projects, Bayer Schering Pharma
09:20	**Is it possible to develop drug development guidelines that encompass HTA requirements?** **Dr Sean Tunis,** Founder and Director, Center for Medical Technology Policy (CMTP), USA
09:40	**How are the needs and dynamics of regulatory agencies changing?** **Dr Thomas Lönngren**, Strategic Advisor, NDA Group, UK
10:00	Break (45 min – to allow for check-out)
Session 6: Feedback from Syndicates	
10:45	**Syndicate One** **Dr Marie-Christine Minjoulat-Rey**, Head, Governance, Global Evidence and Value Development (EVD), sanofi aventis R&D
11:00	**Syndicate Two** **Dr Isaac Odeyemi**, Senior Director and Head of Health Economics and Outcomes Research, Astellas Pharma Europe
11:15	**Syndicate Three** **Dr Mel Walker**, Senior Director, Value Expert Engagement & Collaborations, GlaxoSmithKline
11:30	**Panel Discussion** • **Dr Susan Longman**, Global Head of Cardiovascular/Metabolic Regulatory Affairs, Novartis • **Wim Goettsch**, Deputy Secretary of the Medicinal Products Reimbursement Committee, Dutch Health Care Insurance Board (CVZ) • **Clare McGrath**, Senior Director, HTA Policy, Pfizer • **Seren Phillips,** Associate Director, Scientific Advice Programme, National Institute for Health and Clinical Excellence (NICE)
12:30	**Key outcomes from workshop** **Prof Robert Peterson,** Executive Director, Drug Safety and Effectiveness Network, Canadian Institutes of Health Research
12:50	Final word and close of workshop **Larry Liberti**, Executive Director, Centre for Innovation in Regulatory Science
13:00	Close of workshop