



Workshop on
**New Development Paradigms:
Building Regulatory Confidence for
the Early Release of Medicines**

11 - 12 October 2010

PROGRAMME

**Woodlands Park Hotel,
Surrey, United Kingdom**

CMR International Institute for Regulatory Science Workshop

Background

New Developmental Paradigms; Building Regulatory Confidence for the Early Release of Medicines

As the complexities of pharmaceutical development and the associated costs increase, sponsors and regulatory agencies are seeking novel ways to accelerate patients' access to medicines, while ensuring that a safe and effective product is being made available. Indeed many in the industry and regulatory agencies believe that the linear trial and error model that has been used over the last 20 to 30 years is untenable in the future for the development of a new medicine. A fundamental rethink is required as it is not thought that companies can cost cut their way to sustainability but that there needs to be a more flexible approach to bringing a new medicine to market.

In order to meet this challenge the major Pharmaceutical companies are undertaking different strategies to evolve new development paradigms that will provide a sustainable future. This has seen companies increasing their investment in the emerging markets, become involved in collaborative efforts, improving their understanding of the molecular basis of disease, looking to new technology and genomics as well as more targeted and patient centric drug development. As a result companies are not only refocusing their pipelines, but are also making fundamental decisions in terms of the discovery and development process.

Over the last 5 years the Institute workshops have suggested a number of approaches to reducing time and cost, including the need for consideration of developing early release strategies that can offer safe medicines while establishing their full therapeutic profile and cost benefit. Regulatory agencies have focused on improving the current models and pathways of review rather than looking for radical new models. It has been identified that new measures for post approval monitoring for safety need to be adopted if early release is to become a realistic alternative. This has seen the evolution of risk management plans and Risk Evaluation Mitigation Strategies (REMS). In addition mechanisms are in place for the early release of certain types of medicines such as cancer therapies under controlled conditions in order to test the medicine in the real world. Now the question is what would be needed, pre- and post-release to provide confidence to the regulators of this approach for a wider set of medicines?

Workshop Objectives

- **Identify current companies' and agencies' perspectives** on the need for a development and review process which has the flexibility to enable early controlled real world patient access to medicines
- **Discuss different strategies for different medicines** and their potential consequences for both regulators and Industry
- **Recommend possible new development and review approaches** and what is required pre- and post release to enable regulators to have the confidence in new models being developed with a focus on early release mechanisms

Venue

The Workshop will take place at the Woodlands Park Hotel, Surrey, UK commencing at 09:00 on Monday, 11 October and finishing 13.00 pm on Tuesday, 12 October 2010.

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.

Day 1: Monday 11 October 2010

08:30 Registration

SESSION 1: EVOLVING FLEXIBLE DEVELOPMENT AND REVIEW STRATEGIES: WHAT ARE THE REGULATORY CONSEQUENCES PRE AND POST RELEASE?		
09.00	Chairman's welcome and introduction	Prof Trevor Jones , Director, Allergan Inc, USA
	Is there a need for a more flexible approach to both drug development and review strategies for the medicines of the future?	
	<i>Is the current paradigm fit for purpose for development and regulatory pathways? Is the concept of early release and testing in a real world setting the key to better understanding for regulators, industry and payers of the benefits and risks prior to full release? What are the pre and post release consequences for companies and agencies? What is the role for collaboration between different stakeholders? Can any changes be used to stimulate innovation?</i>	
09.10	Regulatory Viewpoint	Thomas Lönngren , Executive Director, European Medicines Agency
09.35	Industry Viewpoint	Dr Thomas Unger , Executive Director, Worldwide Regulatory Strategy, Pfizer Inc, USA
09.55	Discussion	
10.00	NEWDIGS Initiative – A Regulator's Perspective on New Drug Development Paradigm	Dr John Lim , Chief Executive Officer, Health Science Authority, Singapore
10.20	Discussion	
10.30	Break	
	Post Marketing strategies for identifying benefit and risk – Risk Management Plans: Are these providing the reassurance agencies require and how should benefit be measured?	
	<i>It has been identified that new measures for post approval monitoring for safety need to be adopted if early release is to become a realistic alternative. This has seen the evolution of risk management plans and Risk Evaluation Mitigation Strategies (REMS). However there is also a view that benefit changes should also be measured? Are the and study designs in place to identify benefits downstream, and where how do these fit in early release scenario's?</i>	
11.00	Regulatory Viewpoint	Prof Hans-Georg Eichler , Senior Medical Officer, EMA
11.20	Industry Viewpoint	Moira Daniels , Global Head Regulatory Policy Intelligence and labelling, AstraZeneca, UK
11.40	Discussion	
11.50	Creating an evolving evidence base to enable early release models Healthcare Databases – Are these ready to be harnessed for assessing post-marketing benefit and risks?	Dr Michael Devoy , Head of Global Medical Affairs and Pharmacovigilance, BayerSchering Pharma AG, Germany
	<i>Where are we in the development of databases that can be used in the post marketing phase? Do these have value in an early release scenario? Are regulators and industry confident in what these database can provide? And if not what needs to be done for this to occur?</i>	
12.20	An alternative model to enable early patient access: Proposal for an orphan drug and implications for real world data <i>A worked example for a new therapy</i>	Dr Tony Hoos , Senior Vice President, European Medical Affairs, GlaxoSmithKline, UK
12.45	Discussion	
13.00	Lunch	

SESSION 2: SYNDICATE SESSIONS		
14.00	Summary of the key points from Morning Session	Chairman
14.20	Introduction to the Syndicate Sessions	Dr Neil McAuslane CMR International Institute for Regulatory Science
14.30	<p>Syndicate sessions on</p> <p>TOPIC A: Early release paradigm – What are the scientific and regulatory components that need to be in place post-release for this to become a reality beyond oncology/niche medicines?</p> <p>What models could be envisaged for controlled release? How to balance early market access to new drugs with the need for benefit/risk data ? What are the acceptable trade offs that need to be considered? What should be the criteria/conditions for early release and How should these be managed post release?</p> <p>TOPIC B: What are the implications, both positive and negative, for the various stakeholders (companies, regulators, patients, healthcare providers, payers) in an early release paradigm?</p> <p>Will this stimulate innovation for companies, What do patients need to know and how should this be communicated? Who has liability?, Is the risk shared? Implications for a therapy that needs to be withdrawn? What is in it for the payers? Should companies be reimbursed? What are the potential unintended consequences?</p> <p>TOPIC C: Early release paradigm – What are the scientific and regulatory components that need to be in place pre-release for this to become a reality beyond oncology/niche medicines?</p> <p>What models could be envisaged for controlled release? How to balance early market access to new drugs with the need for benefit/risk data ? What are the acceptable trade offs that need to be considered? What should be the criteria/conditions for early release and How should these be managed post release?</p>	<p>Chairperson: Dr Supriya Sharma, Director General, Therapeutic Products Directorate, Health Canada</p> <p>Rapporteur: Dr Kian Ming Lam, Director, Corporate Development and Operations Division, Health Sciences Authority, Singapore</p> <p>Chairman: Prof Hubert Leufkens, Chairman, Medicines Evaluation Board, The Netherlands</p> <p>Rapporteur: Mark Hope, Head of EU/ROW Program Management and EU/ROW Head of Oncology, F. Hoffmann-La Roche Ltd, Switzerland</p> <p>Chairman: Dr David Jefferys, Senior Vice President, Global Regulatory and Government Relations, Eisai Europe Ltd, UK</p> <p>Rapporteur: Dr Steve Caffè, Senior Vice President, Global Regulatory Affairs and Pharmacovigilance, Baxter Healthcare Corporation, USA</p>
16.00	Break	
16.30	Syndicate resumes	
18.30	End of Syndicate Discussion and end of day one	
19.00	Reception	
19.30	Dinner	

DAY 2: Tuesday 12 October 2010

SESSION 3: PRE AND POST MARKET CLINICAL RESEARCH: HOW DO THESE NEED TO EVOLVE TO ENABLE AN EARLY RELEASE PARADIGM?		
08.30	Chairman's Introduction	Prof Robert Peterson , Executive Director, Drug Safety and Effectiveness Network, Canadian Institutes of Health Research
08:35	Feedback of day one syndicate discussion	
09.15	Panel Discussion: Early release models – Reflection on the syndicate recommendations <i>As companies and agencies work through how to regulate new therapies and new development pathways, dialogue between companies and agencies and between agencies will be essential for acceptance of any changes to the traditional view of development. However there other stakeholders in the today's and tomorrows development including Patients, Health Technology Assessment, Payers etc. What is the right level of co-operation and how can these parties play their part in ensuring any early release model meets their needs?</i>	Margaret Jackman , Group Manager, MHRA Prof Hans-Georg Eichler , Senior Medical Officer, EMA Dr Mary Baker , President, European Federation of Neurological Associations Dr Richard Barker , Director General, ABPI
10.15	Discussion	
10.30	Break	
11.00	Lessons learnt from “Live license” or life cycle regulatory management for oncology /niche medicines – How could these be translated to a wider set of medicines? <i>A cumulative approval process based on gradual accumulation of data. Conditional/orphan approvals can be seen as one potential example, and have been adopted by FDA, EMA, MHRA and France, but could and should this route be envisaged for a wider set of medicines?</i>	Dr Eric Abadie , Chairman, CHMP/EMA, France
11.25	Early Access Scenarios – the Patient Perspective on the concept <i>Early access models have both benefits and Risks for Patients so what is their view on the types of medicines such schemes should be available for and what should be the key criteria?.</i>	Dr Mary Baker President, European Federation of Neurological Associations
11.50	Discussion	
12.00	Early Access Models: What is the perception of those organisations responsible for health technology assessment? <i>What role can a HTA agency have in early access models? Is coverage with evidence development a viable route for some medicines and some patient groups? Are early access models a route to encourage interactions between licensing bodies and HTA agencies as companies need to design confirmatory studies to meet both groups endpoints? How should reimbursement be linked to early access models? Is there a workable model for early (conditional) reimbursement to ensure that early access granted by regulators would translate into early access to patients?</i>	Meindert Boysen , Programme Director Technology Appraisals, NICE
12.25	Discussion	
12.40	Summary of key outcomes and possible next steps	
13.00	Close of Workshop followed by lunch	

