Expediting Patient Access to Medicines: Barriers and Solutions to Simultaneous Submissions and Approvals

Workshop 30 - 31 March 2009

Final Programme

Woodlands Park Hotel,
Cobham, Surrey, UK

CMR International
INSTITUTE FOR REGULATORY SCIENCE

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INSTITUTE FOR REGULATORY SCIENCE

CMR International Institute for Regulatory Science Workshop

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Background

The ICH ultimate goal is to provide companies with harmonized technical requirements for the major markets which would enable simultaneous submissions and regulatory approvals in the world's major markets. Over the last five years the major companies are engaging in global development programmes and have reduced the time between dossier submissions in Europe and the USA and are currently trying to reduce development time not only to Japan but also other markets worldwide. Ideally companies are looking for a global development programme which will lead them to simultaneous submissions and approvals. However this is juxtaposed by an increased barrier to submission where companies have a perception of approval delays, rising requests for further clinical data, and greater chances of rejection or differences in outcomes either from expectation or between agencies.

The utilisation of new technologies which have the potential to better define patient populations, the efficacy and safety parameters for new drugs it is hoped enable a more predictable regulatory approval process and outcome. Companies and agencies are addressing individually and through initiatives such as IMI in Europe and Critical Path in the US, how to improve the efficiency and quality of clinical research undertaken. Ideas which are being utilised to a greater/lesser extent are innovative trial designs, use of new technologies/biomarkers and pharmacogenomics. The question is how are these initiatives progressing and will they enable companies in reaching their objectives of developing globally towards simultaneous submissions. The Workshop will look at the practicalities of achieving simultaneous submission following a global development programme and the barriers to achieving this as well as potential solutions.

Objectives

- **Submission and approval:** To review the current development and submission strategies of companies and the approval performance of the major agencies;
- **Advancements in Clinical Development:** To discuss which new approaches or technologies are an aid towards global development and improving regulatory outcomes.
- **Improving outcome**: To discuss the barriers and how to achieve simultaneous submissions, approvals and predictable outcomes and whether the solution lies in innovative clinical development or relates to a better understanding of the review process and procedures and how benefit & risks are perceived

Outline

This workshop will bring together experts from both regulatory agencies and pharmaceutical companies for a constructive discussion on how companies and agencies are approaching the development and review of new medicines. The first day will explore the current reality of companies achieving simultaneous submission and approval and what are the barriers and solutions. On day two the workshop will explore both agency and company activity in developing new approaches to clinical development and how this may affect global development. All delegates will be invited to participate in the Syndicates in which free-ranging discussions of the issues will be encouraged and possible areas identified for follow-up action and activities.

Date and Venue

The Workshop will take place at the Woodlands Park Hotel, Surrey, England, UK, commencing at 09.00 hrs on 30 March and finishing at 14.00hrs on 31 March 2009.

Style and Participation

Following the agreed practices for Institute Workshops, the meeting will be closed and the size will be limited to a maximum of 50 delegates in order to allow productive networking and discussions. Industry participation (excluding speakers) is restricted to members of the Institute. Senior members of regulatory agencies worldwide are invited to participate both on the programme and as delegates. The summary report prepared immediately after the Workshop will, initially, be made available only to delegates and later to all Institute members and Regulatory contacts. Wider, subsequent reporting of the outcome will be subject to clearance by the Institute executives.

Workshop Programme

DAY 1: MONDAY 30 MARCH 2009

08.30 hrs: **Registration**

SESSION 1:	**GLOBAL DEVELOPMENT AND SIMULTANEOUS SUBMISSION: WHAT IS THE REALITY?**	
09:00	**Chairman's Introduction**	**Prof Hans-Georg Eichler,** *Senior Medical Officer, EMEA, UK*
09.20	**Regulatory approval, submission strategies and roll out time to major markets 1997-2007: What does the data tell us about changes over time?** *Looking at submissions and approvals at FDA, EMEA, TGA, HC and Swissmedic 1997-2007. What does this data tell us about country of first submission, change in approval times, regulatory review time and company time over this period? Also are companies reducing time to submissions in these key markets?*	**Dr Franz Pichler,** *Portfolio Manager, CMR International Institute for Regulatory Science*
10.00	**Advantages and disadvantages to agencies of simultaneous or sequential submissions of a dossier by companies to an agency: An agency viewpoint** *Are there any advantages to an agency to be reviewing a dossier in the same time frame as other agencies? Does this give opportunity for sharing information, issues or the actual review? For submissions that are received following approval at an other agency, what additional factors need to be considered (other agency decision, post-marketing experience etc)?*	**Dr Supriya Sharma,** *Director General, Therapeutic Products Directorate, Health Canada*
10:25	**Discussion**	
10:35	**Break**	
11.00	**Developing Globally, Registering Locally: What are the barriers which impede simultaneous submission and possible solutions?** *Companies are looking today to develop new innovative medicines globally to a common standard which meets the major agency requirements and to reduce time to submit and be approved in the major markets. What are the key barriers and how best can these be overcome? Are the solutions all in the development planning or can agencies enable companies who are developing globally not to be impeded locally?*	**Robin Evers**, *Vice President and Head of Global Regulatory Affairs for Europe, Middle East & Africa, Wyeth Europa, UK*

	Developing Globally, Registering Locally: Same data, similar time frame but different regulatory outcomes – Why and how to mitigate against divergent outcomes? *A number of new medicines are submitted to agencies within a similar time frame but there are divergent outcomes. Why the difference? Is this difference related to different datasets being submitted, different views on how the product has been developed or just down to differences in medical practice/culture? What can companies learn from such events?*	
11.25	**Examples of Divergent Outcomes: US versus EU**	**Dr Paul Huckle**, *Senior Vice President, Global Regulatory Affairs, GlaxoSmithKline, USA*
11.50	**Case Study: Industry Viewpoint**	**Tracy Baskerville**, *Head, Global Regulatory Affairs, Liaison, Cardio-Metabolic, Solvay Pharmaceuticals, France*
12:10	**FDA Viewpoint**	**Dr Murray Lumpkin,** *Associate Commissioner, International and Special Programs, FDA USA*
12:30	**Discussion**	
13:00	**Lunch**	
SESSION 2 : SYNDICATE DISCUSSIONS:		
14:00	**Chairman's Introduction to the Syndicate discussions**	**Professor Robert Peterson,** *Clinical Professor of Paediatrics, University of British Columbia Faculty of Medicine, Canada*
14:15	**Syndicate discussions** **SYNDICATE TOPICS: OPTIONS** To Identify the potential barriers and solutions to simultaneous submission and approval by western and Japanese agencies? To discuss reasons for divergent opinions based on same data, same timeframe, same dossier and how to mitigate the risk?	All participants
15:30	**Break**	
16:00	**Syndicate resumes**	
18:00	**End of Syndicate Discussions & end of day one**	
19:00	**Reception**	
19:30	**Dinner**	

DAY 2: TUESDAY 31 MARCH 2009

SESSION 3:	SOLUTIONS TO ENABLE SIMULTANEOUS SUBMISSION, APPROVAL AND OUTCOMES AND THE ROLE OF INNOVATIVE CLINICAL DEVELOPMENT	
08:30	**Chairman's Introduction**	**Professor Robert Peterson,** *Clinical Professor of Paediatrics, University of British Columbia Faculty of Medicine, Canada*
08:35	**Feedback of Day One Syndicate Discussion**	Syndicate Rapporteurs and Chairpersons
09:35	**Discussion**	
09:55	**Innovative clinical development: What new science opportunities are there and which areas do companies believe have the most promise?** *New technologies, new clinical trial designs, simulation and modelling are just a few of the areas which have been put forward in relation to improving success rates for clinical studies. How are these areas progressing and what are the innovative approaches on the horizon?*	**Dr Damian O'Connell,** *Executive Director, Clinical Group Head, Pfizer Clinical R&D, UK*
10:20	**Break**	
	Genomics, Biomarkers and surrogate endpoints: Are these the key to improving regulatory decision-making based on clinical trials? *It has been suggested that the key to more efficient and informative clinical research is in the utilization of genomics, biomarkers and surrogate endpoints but do they improve regulatory decision making? Is the use of these new approaches moving forward in the way originally envisaged? If progress is slower than anticipated, is this due to scientific hurdles or is there a need to* ***re-think regulatory approaches?***	
10.50	**Industry Perspective**	**Alison Lawton,** *Head of Global Regulatory Affairs, Genzyme Corporation, USA*
11.05	**European Regulatory Perspective**	**Dr Eric Abadie,** *Chair, CHMP, EMEA*
11.20	**TGA Viewpoint**	**Dr Leonie Hunt,** *Head, Office of Prescription Medicines, TGA, Australia*

	Improving clinical development and regulatory outcomes: How can collaborative efforts of the Innovative Medicines and the Critical Path initiative aid companies and agencies? *Two high level collaborative initiatives have been started in Europe and the USA looking either to reduce bottlenecks in the development process or to look at how to identify practices that will increase the quality and efficiency of clinical trials. Both initiatives are looking to improve a better understanding about the benefits and risks of a medical product: What are these initiatives specifically focusing on? And how may they improve the clinical development process?*	
11.40	**Improving Efficacy data: Update on the IMI initiative**	**Prof Trevor Jones,** *Member of the Scientific Committee, IMI*
12.05	**Clinical Trials Transformation Initiative: Update on the CP Initiative**	**Dr Alberto Grignolo**, *Corporate Vice President, Global Strategy and Services PAREXEL Consulting, USA*
12.30	**Discussion and Chairman's Summary**	
13.00	**Close of meeting**	

Syndicate Groups

Topic A: To identify he potential barriers and solutions to simultaneous submission and approval by Western and Japanese agencies		
Syndicate 1	*Chair*: Prof Sir Alasdair Breckenridge, *Chairman, MHRA, UK*	*Rapporteur:* Dr Kathryn Broderick, *Associate Director, Eli Lilly and Company, USA*
Topic B: To discuss reasons for divergent opinions based on same data, same timeframe, same dossier and how to mitigate the risk		
Syndicate 2	*Chair:* Prof Tomas Salmonson, *Vice Chair, CHMP, EMEA*	*Rapporteur*: Dr Victor Raczkowski, *Vice President, US Regulatory Affairs, Solvay Pharmaceuticals Inc, USA*