Workshop

Medicines adaptive pathways:
(Facilitated Regulatory Pathways)
A practical strategy to improve patient access to medicines?

1 - 2 October 2014

PROGRAMME

Venue:
Heathrow Windsor Marriott Hotel, UK

CENTRE FOR INNOVATION IN REGULATORY SCIENCE
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Organisers

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10 Sept 2014

Centre for Innovation in Regulatory Science Workshop

Medicines adaptive pathways (Facilitated regulatory Pathways): A practical strategy to improve patient access to medicines?

Background

Different approaches to meet unmet medical needs have been adopted by regulatory agencies for making new medicines available to patients more rapidly. The advantages of these models are:

- A streamlined regulatory review process enhanced by more frequent/formal sponsor-agency interactions during development - More rapid availability to patients than standard approaches
- The possibility to integrate the scientific requirements of HTA and payers into the development process
- The use of innovatively designed studies incorporating predictive endpoints to aid in answering questions around benefits, harms and effectiveness of the new medicine with smaller study cohorts than previously assessed
- Ability to address regulatory uncertainty associated with an early release model through the collection of real world data
- The ability to manage the product in the post-approval period including implementing ways to reduce its availability if benefits and harms are not as expected in early development

It is, therefore, important that these models dynamically assess the benefits and harms in the post-approval phases. For these regulatory pathways to successfully deliver safe and effective new medicines to patients quicker, then it is also necessary for the HTA or reimbursement bodies to be an integral part in the development and acceptance of any new approaches.

This workshop will build on previous CIRS workshops where recommendations to advance this concept have been identified. This workshop will do more than just provide the current overview of the different medicines adaptive pathways being discussed, designed, piloted and implemented around the world. Rather, it will provide perspectives on the opportunities and hurdles from the points of view of the sponsor, the HTA and the licensing bodies and will focus on specific building blocks needed to refine and implement these paradigms. The focus of this workshop will be to:

- Consider whether new approaches to medicines availability are needed and how will they be valued by companies, patients, HTA and licensing agencies;
- Identify the commonalities among the various medicines adaptive pathways and related Facilitated Regulatory Pathways and to explore common elements in detail;
- Explore which new methodologies, including novel clinical designs that are being considered and what the opportunities and challenges are to different stakeholders;

Workshop Objectives

- Discuss the **new regulatory approaches to accelerating medicines availability** for patient use and the role of HTA and coverage bodies in enabling access
- Make recommendations on how best to ensure success of **new Facilitated Regulatory Pathways** and what will be their **critical success** factors to manage uncertainty, ensure proper use, and to interpret continuity with evidence generated during early phases of study.
- Identify the **possible pathways for an integrated approach which are acceptable** to all stakeholders for adaptive routes and discuss the challenges and opportunities for **the regulatory and HTA groups and the sponsor.**

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.

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Day One: Wednesday, 1st October 2014

08:30 Registration

SESSION 1: MEDICINES ADAPTIVE PATHWAYS: WHAT ARE THE CRITICAL ELEMENTS, CHALLENGES AND OPPORTUNITIES?		
09:00	**Chair's welcome and introduction**	**Prof Hans-Georg Eichler,** Senior Medical Officer, European Medicines Agency
09:10	**Are new facilitated approaches to medicines availability needed and what are the major challenges? Perspectives from key stakeholders (patients, companies, HTA and licensing agencies)**	**Larry Liberti,** Executive Director, CIRS
09.30	**Discussion**	
	What approaches or initiatives are used or being considered by Regulatory and HTA Agencies to provide flexible and/or adaptive pathways to gain regulatory approval and reimbursement?	
09:35	**EMA Framework development and Pilot Study for Adaptive Licensing: What are the key considerations and aspirations?**	**Prof Tomas Salmonson**, Chair, CHMP, European Medicines Agency
09:55	**Building flexibility for regulatory approval in the US. What is being used or considered and what are the perceived advantages and barriers?**	**Dr Amy Egan,** Deputy Director, Office of New Drug Evaluation III and Acting FDA Liaison to European Medicines Agency
10:15	**What are the possible pathways to adapt coverage decisions as new evidence comes in: What can be practically considered and what are the main hurdles and possible solutions?**	**Dr Brian O'Rourke**, President and CEO, Canadian Agency for Drugs and Technologies in Health
10:35	**Discussion**	
10:40	**Break**	
11:10	**Adaptive Licensing: Lessons Learned from 4 Years of Evaluation in MIT NEWDIGS**	**Dr Tony Hoos**, Core Member of NEWDIGS & President M4P Consulting, UK
11:30	**What would practical study designs look like in generating the evidence for the initial approval and how should these be linked to post-approval evidence generation - How do we know when we can be confident of a "predictive endpoint"?**	**Dr Donald Berry,** Professor, Department of Biostatistics, University of Texas Anderson Cancer Center, USA
11:50	**Case Study: Adaptive trial designs, or other 'seamless' ways of ensuring continuing generation of evidence**	**Dr Mark Higgins,** Senior Clinical Director, CF, Vertex Pharmaceuticals UK
12:10	**Discussion**	
	How can uncertainty be managed in practice to meet the needs of different stakeholders?	
12:15	**Agency Perspective**	**Kelly Robinson,** Director, Bureau of Metabolism, Oncology and Reproductive Sciences, Health Canada
12:35	**HTA Perspective**	**Dr Wim Goettsch**, Advisor, National Health Care Institute, The Netherlands
12:55	**Discussion followed by lunch**	

Day 1: Wednesday 1st October 2014

SESSION 2: MEDICINES ADAPTIVE PATHWAYS: WHAT ARE THE MAIN BUILDING BLOCKS AND PRACTICAL HTA AND REGULATORY STRATEGIES FOR ADOPTION?

14:00	**Introduction to session**	
	What sort of decision (withdrawal and exit strategies, adaptive disengagement, orchestrated safeguards) is required when the post-approval evidence does not support the initial potential?	
14:05	**Agency Perspective**	**Dr Almath Spooner,** Pharmacovigilance and Risk Management Lead, Health Products Regulatory Agency, Ireland
14:25	**HTA Perspective**	**Dr Andrew Mitchell,** Strategic Adviser, Evaluation, Department of Health and Ageing, Australia
14:45	**Company Perspective**	**Dr Indranil Bagchi**, Vice President and Head, Payer Insights and Access, Global Health and Value, Pfizer Inc, USA
15:05	**Discussion**	
	Case Studies	
15:10	**How should a company address opportunities to use facilitated regulatory approaches?**	**Merete Schmiegelow,** Senior Director, Regulatory Advocacy, Novo Nordisk, Denmark
15:30	**How do HTA use models and simulation to extrapolate efficacy data and how these could be used effectively in a facilitated or adaptive pathway?**	**Meindert Boysen,** Programme Director, Technology Appraisals National Institute for Health and Care Excellence
15:50	**How do patients perceive early access schemes and adaptive licensing approaches – with hope or concern?**	**Alastair Kent,** Chair of Rare Disease UK and Director of Genetic Alliance UK
16:10	**Discussion**	
16:15	**Introduction to Syndicates Break**	
	Syndicate sessions - *Each syndicate will undertake the following using a structured format to address the syndicate topic. Based on a proposal and a set of questions or outline, the syndicate is asked to review, debate and make recommendations to answer the question.* • Syndicate 1: Managing uncertainty and ensuring appropriate utilisation post initial approval - Are the systems in place? ***Chairperson****: Prof Angela Timoney, Director of Pharmacy, NHS Lothian* ***Rapporteur****: Jesús Muñiz, Senior Director, Regulatory Policy and Intelligence, Shire, USA* • Syndicate 2: What type of management plans need to be developed for implementation at the front end to manage questions that arise post initial approval? ***Chairperson:*** *Dr Andrew Mitchell, Strategic Adviser, Evaluation, Department of Health and Ageing, Australia* ***Rapporteur****: Dr Michiel Hemels, Director, EMA HEMAR, Janssen, Denmark* • Syndicate 3: Incentives for using an facilitated approach: What are they and how can this best be achieved? – Company, HTA, Regulatory perspective ***Chairperson****: Barbara Sabourin, Director General, Therapeutic Products Directorate, Health Canada* ***Rapporteur****: Andrew Storey, Vice President, Regulatory Affairs, US/Canada, AbbVie, USA*	
18:00	**End of day one**	
19:00	**Reception and workshop dinner**	

DAY 2: Thursday 2nd October 2014

SESSION 3: CRITICAL SUCCESS FACTORS FOR ADAPTIVE PATHWAYS AND HOW CAN THIS PRINCIPLE BE USED TO FACILITATE MEDICINES AVAILABILITY?		
08:30	**Syndicate sessions resume: Agreement of recommendations**	
10:00	**Syndicate sessions end**	
10:40	**Chairman's Introduction**	**Prof Richard Barker,** Director, CASMI
10:45	**Feedback of syndicate discussion and participants viewpoint**	
	Is there a viable commercial strategy for the use of adaptive approaches, now and in the future? *What do HTA need to see in order to provide a decent added benefit rating so that industry can achieve appropriate prices and be incentives to use an adaptive approach*	
11:45	**Academic perspective**	**Prof Adrian Towse**, Director Office of Health Economics, UK
12:05	**Company Perspective**	**Dr Jens Grueger**; Vice President, Global Pricing and Market Access, F. Hoffmann-La Roche, Switzerland
12:25	**Discussion**	
12:30	**UK early access Scheme: What is it and how will it work?**	**Dr David Jefferys,** Senior Vice President, Eisai, UK
12:50	**Discussion**	
13:00	**Lunch**	
14:00	**How to move from current mindset to an "adaptive mindset": what do companies, regulatory agencies, HTA and Payers need to adopt?**	
	HTA Perspective	**Prof Sarah Garner**, R&D Associate Director, National Institute for Health and Care Excellence
	Regulatory Perspective	**Prof Alasdair Breckenridge**, Former Chairman, MHRA
	Company Regulatory Perspective	**Sharon Olmstead,** Global Head, Development and Regulatory Policy, Novartis Pharmaceuticals, USA
	Company HTA Perspective	**Dr Eric Giesen,** Director, Market Access Policy, Bayer Pharma AG, Germany
15:00	**Discussion**	
15:15	**Summary**	
15:30	**Close of Workshop**	