Annual Benefit-Risk Workshop

Assessment in the Post-Approval Period: How to ensure a life cycle approach to evaluating benefits and risks

12- 13 June 2014

PROGRAMME

Venue: The Sofitel Hotel Washington, DC, USA

CENTRE FOR INNOVATION IN REGULATORY SCIENCE
The Johnson Building, 77 Hatton Garden, London EC1N 8JS, UK, Telephone: +44 (0) 207 433 4247 Email: ghepton@cirsci.org

Organiser
Professor Stuart Walker: swalker@cirsci.org
Neil McAuslane: nmcauslane@cirsci.org
Lawrence Liberti: lliberti@cirsci.org

14 March 2014

Centre for Innovation in Regulatory Science Workshop

Background

Assessment in the post-approval period: How to ensure a life cycle approach to evaluating benefits and risks

One of the criteria for the development of a framework to assess benefits and risks is that it provides the same standardized structured systematic approach to the assessment of the benefits and risks throughout the entire life cycle of medicines development. This will lead to not only enhanced documentation and communication of the changing benefit risk profile of medicines, but also to identify or evolve appropriate methodologies to measure benefits in the post marketing period.

To date, this has stimulated the FDA, EMA, and the four agency consortium (Swissmedic, Health Canada, Australian TGA and HSA in Singapore) as well as companies to evaluate and produce qualitative frameworks. An evaluation of these initiatives demonstrates that although each agency may come from a different perspective, what has been achieved is consistency in the principles of the requirements to describe a benefit risk assessment. This has led to the creation of an overarching framework, or UMBRA (Universal Methodology for Benefit Risk Assessment).

The importance of the post-approval period in providing a better understanding both of the benefits and harms of medicines has been reflected in the recent ICH E2 guideline which now requires companies to provide a structured benefit risk evaluation within the PBRERS and PSURS.

The discussion at this workshop will be around how utilising an overarching framework which covers pre, peri and post approval can enable an improved understanding of the changing benefit risk profile as knowledge increases about a new medicine. Structured approaches to evaluating the evidence in balancing benefit-risk in the post-approval period:, what are the challenges, the hopes and expectations will also be explored along with what methodologies would be feasible for companies and acceptable to agencies to provide information on the benefits of a new medicine in the post marketing period?

This workshop will also provide an update on the various regulatory methodologies to assess benefits and harms with a focus specifically on both company and agency experience of using a structured approach and how this can translate to the post-marketing phase.

Workshop Objectives

- **Discuss how a** universal framework aligns to the structured benefit risk assessment of medicines across the pre, peri and post approval periods.
- **Identifying appropriate** methodologies for Producing Periodic Benefit Risk Evaluation Reports (PBRERS) - Challenges and solutions
- **Make recommendations** on how best to assess benefits in the post approval period.

Venue

The Workshop will take place at The Sofitel Hotel in Washington DC, commencing at 09:00 on 12th June and finishing with lunch on the 13th June 2014

Style and Participation

Following the agreed practices for Institute Workshops, the meeting will be closed and attendance will be limited to allow productive networking and discussions.

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Day 1: 12th June 2014

08.30 Registration

SESSION 1: UTILISING A BENEFIT-RISK FRAMEWORK: HOW ARE AGENCIES MODIFYING THEIR REVIEW PROCESS AND HOW WILL THIS ENABLE POST-APPROVAL ASSESSMENT?		
09:00	**Chair's welcome and introduction**	**Prof Tomas Salmonson,** Chair, CHMP, European Medicines Agency
	Does requiring a structured approach to benefit-risk assessment in the post-approval period drive the need for consistent methods in the approval period?	
09:20	**FDA viewpoint**	**Dr Theresa Mullin,** Director, Office of Strategic Programs, CDER, Food and Drug Administration
09:45	**Industry viewpoint**	**Dr Paul Huckle,** Chief Regulatory Office, GlaxoSmithKline, USA
10:10	**Discussion**	
	Benefit-Risk Framework development: Current status and forward plans *Initiatives to develop a Benefit-Risk Framework are ongoing at FDA, EMA, the consortium of four agencies and across companies. This session will provide an understanding of the main internal and external challenges, current status and forward plans.*	
10:15	**FDA Framework development and testing**	**Patrick Frey,** Director, Office of Program and Strategic Analysis, CDER, Food and Drug Administration
	Discussant – Using the framework	**Kimberly Witzmann,** Medical Officer, Division of Pulmonary, Allergy and Rhematology Products, Office of New Drugs, Food and Drug Administration
10:45	**Break**	
11:15	**EMA Framework development and Pilot Study**	**Dr Franceso Pignatti,** Head of Section, Oncology, Haematology & Diagnostics , European Medicines Agency
11:35	**An evaluation of the application of UMBRA to ensure a systematic documentation of benefit-risk in non ICH countries**	**Dr Neil McAuslane,** Director, CIRS
11:55	**The utilisation of the summary template for benefit-risk assessment of medicines by HSA**	**Dr James Leong,** Senior Regulatory Specialist Health Sciences Authority, Singapore
12.15	**Discussion**	
12.30	**Lunch**	

Day 1: 12th June 2014

SESSION 2: BENEFIT-RISK DECISION MAKING IN THE POST-APPROVAL PERIOD: HOW IS THIS BEING APPROACHED AND WHAT NEEDS TO BE CONSIDERED?		
13:30	**Chairman's Introduction**	**Prof Tomas Salmonson,** Chair, CHMP, European Medicines Agency
13:35	**Issues in measuring benefit-risk in the post-approval period: What are the challenges for regulatory agency acceptance?**	
	Agency Viewpoint	**Dr Co Pham,** Senior Science Advisor, Marketed Products Directorate, Health Canada
	Company Viewpoint	**Dr Stephen Knowles,** Senior Director, Global Patient Safety, Eli Lilly and Company
14:05	**New approaches/technologies to capture benefits and risks in the post-approval phase – What are the practical and regulatory challenges?**	
	FDA Viewpoint	**Dr Gerald Dal Pan,** Director, Office of Surveillance and Epidemiology, Food and Drug Administration, USA
	Company Viewpoint	**Dr Carmen Bozic,** Senior Vice President, Clinical and Safety Sciences, Biogen Idec, USA
14:35	**Discussion**	
	Recommendations for measuring benefit-risk in the post-approval space	
14:45	**PMDA Perspective**	**Dr Akiko Hori,** Director, Office of Safety II, Pharmaceutical and Medical Devices Agency, Japan
15:00	**IMI PROTECH initiative**	**Prof Deborah Ashby**, Co-Director of Imperial Clinical Trials Unit and Deputy Head, School of Public Health, Imperial College London, UK
15:15	**Discussion**	
15:30	**Break**	

Day 1: 12th June 2014

16:00	**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.* **Topic A: Collection of benefits and harms in the post-approval period: What are future methodologies** **Chair: Prof Robert Peterson,** Executive Director, Drug Safety Effectiveness Network, Canadian Institute of Health Research **Rapporteur: Anders Lindholm,** Pharmacovigilance and Risk Management TA Head**,** Shire Pharmaceuticals, USA **Topic B: PBRER'S: What are company's experiences in providing agencies with structured benefit-risk analysis?** **Chair: Prof Bruno Flamion,** Professor of Pharmacology, University of Namur, Belgium **Rapporteur: Dr Leo Plouffe,** Vice President, Head of Risk Management Global Pharmacovigilance, Bayer HealthCare Pharmaceuticals, USA **Topic C: Patient input into the post-approval methods for collection of benefits and harms – what is their role?** **Chair: Dr John Bridges,** Associate Professor, John Hopkins Bloomberg School of Public Health, USA **Rapporteur: Dr Rick Hermann,** Safety Science Physician, AstraZeneca, USA
18:00	**End of Session**
19:00	**Reception**
19:30	**Dinner**

DAY 2: 13th June 2014

SESSION 3: SYNDICATE SESSIONS & FEEDBACK		
08:30	**Syndicate sessions resume**	
10:00	**Break**	
10:40	**Chairman's Introduction**	**Prof Sir Alasdair Breckenridge**
10:45	**Feedback of syndicate discussion and participants viewpoint following each syndicate discussion**	
11:45	**Understanding the benefits, risks and their relative importance to patients: Challenges and recommendations**	
11:45	**Agency viewpoint**	**Dr Theresa Mullin,** Director, Office of Strategic Programs, CDER, FDA
12:00	**Industry viewpoint**	**Dr Bennett Levitan**, Director, Janssen, USA
12:15	**Patient viewpoint**	**Dr Durhane Wong-Rieger,** President, Canadian Organisation for Rare Disorders
12:30	**Communicating benefit-risk decisions to stakeholders**	**Prof Stuart Walker,** Founder, CIRS
12:50	**Discussion**	
12:55	**Summary**	
13:00	**Close of Workshop followed by lunch**	