Annual Benefit-Risk Workshop

Building the Benefit-Risk Toolbox:

Are there enough common elements across the different methodologies to enable a consensus on a scientifically acceptable framework for making benefit-risk decisions?

20 - 21 June 2012

PROGRAMME

The Madison 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

Centre for Innovation in Regulatory Science Workshop

Background

Building the Benefit-Risk Toolbox: Are there enough common elements across the different methodologies to enable a consensus on a scientifically acceptable framework for making benefit-risk decisions

A survey undertaken by CIRS in 2011 identified the biggest barriers to implementing a formal benefit risk framework within companies and agencies as the lack of a scientifically accepted/ recognised framework. This is against a background where there is generally a good agreement on the need and function of an appropriate BR framework as well as the perceived advantages for implementing a framework as a tool for communication, structured discussion and enhancing transparency/ accountability. It is also starting to be realised perhaps rather than looking for one single Benefit-Risk methodology what is required is a toolbox of methodologies that are flexible and adaptable for different situations although for this to be taken forward there still requires a consensus amongst the major stakeholders on a general scientifically accepted framework.

Over the last five years a number of initiatives at regulatory agencies, (EMA, FDA, Four agency consortium), individual companies and across companies (BRAT initiative) have developed Benefit-Risk methodologies, qualitative, semi-quantitative and quantitative all of which have a number of common elements. These initiatives are now undertaking pilot projects to try and apply the models/ methodologies to real world cases.

In 2012, as the development of benefit-risk methodologies move forward through FDA, EMA, BRAT and the consortium of four agencies, this workshop is being designed to bring together the various stakeholders who are or have undertaken pilot projects to test their benefit risk methodologies. Case studies will be presented and discussed in the context of what are the common elements. The question is can the stakeholders agree on a scientifically accepted approach/framework to benefit risk decision making in the registration of new medicines?

.

Workshop Objectives

- **Discussing the progress** made since 2011 by the different groups on defining and implementing a benefit-risk methodology framework within their organizations.
- **Furthering the thinking.** What can be learnt from case studies and from other stakeholders about different methodologies that can be used to making explicit benefit risk decisions?
- **Identifying the common elements** across methodologies and discussing how to achieve a consensus on a scientifically accepted approach for making benefit risk decisions

Venue

The Workshop will take place at the Madison Hotel in Washington DC, commence at 09:00 on Wednesday 20th June and finishing at 17:00 on Thursday 21st June 2012.

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.

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

Day 1: Wednesday 20 June 2012

08:30 Registration

SESSION 1: DEVELOPMENT OF A FRAMEWORK FOR BENEFIT-RISK: WHAT HAS BEEN LEARNT THROUGH CASE STUDIES?	
09.00	**Chairman's welcome and introduction** **Dr Murray Lumpkin,** Commissioner's Senior Advisor and Representative for Global Issues, US Food and Drug Administration
09:05	**The role PDUFA V will play in delivering a benefit-risk framework for FDA by 2013** **Dr Theresa Mullin,** Director, Office of Planning and Informatics, Center for Drug Evaluation and Research, Food and Drug Administration, USA
	Achieving a scientifically acceptable framework for benefit-risk decision making: Should this be based around a toolbox of methodologies underpinned by common elements?
09.15	**Industry Viewpoint** **Dr Tim Garnett,** Chief Medical Officer and Senior Vice President, Eli Lilly and Company, USA
09:35	**Regulatory Viewpoint** **Prof Hans-Georg Eichler,** Senior Medical Officer, European Medicines Agency
09.55	**Discussion**
	Benefit-Risk Framework Development: Case studies and forward plans
10.00	**Four Agency Consortium** **Dr Jason Ferla**, Director, Prescription Medicines Clinical Unit 3, Office of Medicines Authorisation, Therapeutic Goods Administration, Australia
10.30	**Break**
	Benefit-Risk Framework Development: Case studies and forward plans - continued
11.00	**EMA Case Study** **Dr Francesco Pignatti,** Head of Section Oncology Safety and Efficacy of Medicines, European Medicines Agency
11.30	**FDA Case Study** **Patrick Frey**, Director **,** Office of Planning and Analysis, Center for Drug Evaluation and Research, Food and Drug Administration, USA
12.00	**Company Case Study using the BRAT Methodology** **Dr Filip Mussen,** Head, Global Labeling Center of Excellence, Janssen Research and Development, Belgium
12.30	**Academia, Industry and Regulatory Case Study** **Dr Sinan Sarac,** Senior Medical Officer, Danish Health and Medicines Authority
13:00	**Lunch**

Day 1: Wednesday 20 June 2012

SESSION 2: BENEFIT-RISK DECISION MAKING: HOW AND WHEN TO INVOLVE PATIENTS TO HELP INFORM THE BENEFIT-RISK DECISION MAKING OF COMPANIES AND AGENCIES	
14.00	**Chairman's introduction** **Dr Murray Lumpkin,** Commissioner's Senior Advisor and Representative for Global Issues, US Food and Drug Administration
14:05	**Update on the IMI initiative** **Dr Diana Hughes,** Vice President, Worldwide Safety Strategy, Primary Care Business Unit Lead, Pfizer Inc, USA
14.30 *14:50*	**How and when should patients be involved in making benefit-risk decisions** **European patient viewpoint** **Jean Mossman,** Policy Lead, European Federation of Neurological Associations **USA patient viewpoint** **Dr Lucie Bruijn**, Chief Scientist, ALS Association, USA
15:10	**Company viewpoint** **Dr Diana Hughes,** Vice President, Worldwide Safety Strategy, Primary Care Business Unit Lead, Pfizer Inc, USA
15:30	**Patients' perspectives on benefit and risks in drug development** **Dr Theresa Mullin,** Director, Office of Planning and Informatics, Center for Drug Evaluation and Research, Food and Drug Administration, USA
15:55	**Discussion**
16:00	**Break**
16:30	**Decision-Making: what are the challenges in making quality decisions?** **Ronan Donelan,** Head of Regulatory Affairs EMEA and ANZ, Quintiles, Ireland
16:55	**Reflections from a company** **Dr Paul Huckle,** Chief Regulatory Officer, GlaxoSmithKline, USA
17:10	**Reflections from an agency** **Prof Sir Alasdair Breckenridge,** Chairman, Medicines and Healthcare products Regulatory Agency, UK
17:25	**Discussion**
18:00	**End of Session**
19.00	**Reception**
19.30	**Workshop Dinner**

DAY 2: Thursday 21 June 2012

SESSION 3: SYNDICATE SESSIONS	
08.30	**Chairman Introduction** **Dr Frank Rockhold,** Senior Vice President, Global Clinical Safety and Pharmacovigilance, GlaxoSmithKline, USA
08.35	**Methodologies to assess benefit-risk in the context of licensing and in relation to HTA: What can be learnt and do the similarities outweigh the differences? Health Canada and CADTH** **Barbara Sabourin,** Director General, Therapeutic Products Directorate, Health Canada **Dr Chander Sehgal,** Director of the CADTH Common Drug Review program, Canadian Agency for Drugs and Technologies in Health
09.05	**Introduction to the Scenarios and Methodology**
09.10	**Syndicate sessions** Each syndicate will undertake the following using a structured format to answer the syndicate question. Based on a set of questions or outline, the syndicate is asked to review, debate and make recommendations to answer the question. **Syndicate 1: Can there be agreement (alignment) on the various components that should be included in any ideal model/framework?** **Chair: Prof Sir Alasdair Breckenridge,** Chairman, Medicines and Healthcare products Regulatory Agency, UK **Rapporteur: Dr Becky Noel,** Senior Research Scientist, Eli Lily and Company, USA **Syndicate 2: What are the challenges and the processes/procedures which would enable agencies and companies to make quality decisions in benefit-risk assessments?** **Chair: Professor Sam Salek,** Director, Centre for Socioeconomic Research, Cardiff University, UK **Rapporteur: Dr Richard Hermann,** Safety Science Physician, AstraZeneca, USA **Syndicate 3: When and how should patients be involved and what would facilitate their involvement with regard to the benefit-risk assessment of new medicines?** **Chair: Dr Paul Huckle,** Chief Regulatory Officer, GlaxoSmithKline, USA **Rapporteur: Dr Nadine Cohen,** Senior Vice President, Regulatory Affairs, Biogen Idec, USA
12.30	**End of Syndicate Discussions**
12.30	**Lunch**

SESSION 4: CHALLENGES AND DIFFICULTIES OF PRESENTING BENEFIT-RISK INFORMATION TO STAKEHOLDERS – IS ALIGNMENT THE KEY TO INFORMED DECISION MAKING AND INFORMATION SYMMETRY?	
14.00	**Chairman's Introduction**
14:05	**Feedback of syndicate discussion**
15:05	**Break**
15:35	**Panel viewpoint following each syndicate discussion** **Company Representatives:** **Dr Carmen Bozic,** Senior Vice President and Global Head, Drug Safety and Benefit-Risk Management, Biogen Idec, USA **Dr Susan Welsh,** Vice President, Global Pharmacovigilance and Epidemiology, Medical Safety Assessment Therapeutic Area Head - Head, Oncology & Immunology, Bristol-Myers Squibb, USA **Licensing body, Europe and USA representatives;** **Prof Hans-Georg Eichler,** Senior Medical Officer, European Medicines Agency **Dr Theresa Mullin**, Director, Office of Planning and Informatics, Center for Drug Evaluation and Research, Food and Drug Administration, USA **Patient representative:** **Jean Mossman,** Policy Lead, European Federation of Neurological Associations
16.35	**The Benefit-Risk Task Force - what has been achieved and what action is required for the next 12 Months?** **Prof Sir Alasdair Breckenridge,** Chairman, Medicines and Healthcare products Regulatory Agency, UK
16.50	**Summary**
17.00	**Close of Workshop**