



**CMR International
Institute for Regulatory Science**

Executive Forum

**Refining the Benefit-Risk Framework
for the Assessment of Medicines:
Valuing and weighting benefit and risk
parameters**

FINAL PROGRAMME

**17th - 18th June 2010
St Regis Hotel, Washington DC, USA**



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Background

Determining the benefit-risk balance of a medicine is one of the most important steps in its development, review and post-approval re-assessment. The need and establishment of a Benefit Risk framework is recognised as critical from both an agency and a company perspective in order that there is not only a transparent articulation of the benefits and risks considered in making the final decision but by applying the parameters derived from new assessment models, regulatory agencies and sponsors have a framework that allows a scientific discussion of benefit-risk.

There is a consensus of what the essential elements that should be in any framework as well as identification of 5 key steps that take the data through a systematic process prior to making the final decision, these are; construction of summary tables; value tree of benefits and risks; assessment of importance and prioritisation of benefits and risks; Assignment of value and weightings for the benefits and risks; and final benefit risk assessment or expert judgement.

Although there is consensus of the steps that are required for the framework and for the process the assignment of weighting and values is more difficult with there being different views, methods and stakeholders perspective not just on what should be used but how they should be used.

In order to address this specific issue, the CMR International Institute for Regulatory Science based on the workshops held by the Institute in 2007 and 2008 are holding this one day executive forum to focus on assignment of value and weightings to be used in a benefit risk framework in order to help refine the benefit risk framework. The discussion will be facilitated around real case studies.

Objectives

- Provide a forum for regulatory agencies and industry to review case studies and discuss the utilisation of weighting and values in making the benefit risk decision.
- Provide the agency and industry perspectives of what weighting and values can be used and what issues need to be addressed within the benefit risk framework
- Provide the opportunity to refine the benefit risk framework with an agreement on the principles that should be used in the weighting and values step of the framework

Venue

The Executive Forum will take place at the St Regis Hotel, Washington, DC, commencing at 15.30 on Thursday 17th June and finish at 16:30 on Friday 18th June 2010.

THURSDAY 17TH JUNE 2010

15:00 Registration

SESSION 1: BENEFIT-RISK ASSESSMENTS: COMPANY SUBMISSIONS - HOW IS BENEFIT-RISK ASSESSMENT BEING FRAMED		
15.30	Chairman's welcome, introduction and setting the scene	Professor Stuart Walker , Founder, Institute for Regulatory Science
	Presentation of approaches / case studies from pharmaceutical companies using their own benefit-risk framework	
15.50	Eisai's Approach to Benefit-Risk Assessment	Dr David Jefferys , Senior Vice President, Global Regulatory, Healthcare Policy Dept, Eisai Europe Ltd, UK
16.20	Novo Nordisk's Development of a Benefit-Risk Assessment Model	Dr Sinan Bardakcki Sarac , Industrial PhD Student and Dr Christine Hallgreen , IMI PostDoc, Clinical Pharmacology - Biosimulation Novo Nordisk A/S, Denmark
16.50	Visualisation of Benefits and Risks – an innovative approach?	Dr Douglas Manion , Vice President, Neuroscience and Virology, Bristol-Myers Squibb, USA
17.20	Discussion	
17.45	Conclusions	Professor Stuart Walker , Founder, Institute for Regulatory Science
17.50	Close of Day One	
19:00	Reception	
19.30	Dinner	

FRIDAY 18TH JUNE 2010

SESSION 2: BENEFIT-RISK ASSESSMENTS: COMPANY SUBMISSIONS HOW IS BENEFIT RISK ASSESSMENT BEING FRAMED		
08.30	Chairman Introduction and recap of first day	Prof Sir Alasdair Breckenridge , Chairman, MHRA
Focus on Weighting and Valuing in A Benefit Risk Framework		
08.45	A Case Study from Eli Lilly	Dr Rebecca Noel , Research Scientist, Eli Lilly and Company, USA
09.15	A Case Study from GSK	Dr Marilyn Metcalf , Director, Quantitative and Decision Sciences, GlaxoSmithKline, USA
09.45	General Discussion	
10.00	Break	
Agency views		
10:30	Update from EMA on benefit-risk assessment	Dr Xavier Luria , Head of Safety and Efficacy of Medicines, EMA
10:55	Regulatory Decision Maker's Perspective on Benefit Risk Considerations	Dr Theresa Mullin , Director, Office of Planning and Informatics, CDER, FDA
11:20	Discussion: Reflections from Agencies and Companies on valuing and weighting of benefit-risk parameters	Discussion Led by Chairman
12.00	A Scenario for Discussion: Setting the Scene <i>A scenario has been developed for the evaluation of a hypothetical statin in comparison with no treatment. This will be presented from the viewpoint of how a benefit-risk assessment might be evaluated. Then during the syndicate sessions after lunch, there will be the opportunity for three individual groups representing patients, sponsors and the regulatory authorities to develop a value tree of benefits and risks, to provide a ranking for weighting and then to make final benefit-risk assessment from the viewpoint of the relevant stakeholders</i>	Dr Bennett Levitan , Director, Quantitative Safety Research, Johnson & Johnson PRD, USA
12.45	Lunch	
13.45	Syndicate session for the statin scenario exercise <i>Participants will be divided into three groups representing the regulatory authorities, the pharmaceutical industry sponsors and patients and these three syndicate groups will be facilitated by Dr Paul Coplan, Dr John Ferguson and Dr Rebecca Noel</i>	All Participants
15:15	Break	
15:30	Feedback from the syndicate sessions and summary of key points and recommendations with regard to the appropriate methodology for valuing and weighting of benefit and risk parameters	
16.30	Close of meeting	