Benefit-Risk Framework for the Assessment of Medicines:

Valuing the Options and Determining the Relative Importance (weighting) of Benefit and Risk parameters

Technical Workshop

13 December 2012

Sofitel, Philadelphia, USA

CENTRE FOR INNOVATION IN REGULATORY SCIENCE
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Organiser
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Centre for Innovation in Regulatory Science Workshop

Background

In 2012 at the CIRS annual workshop there was an agreement from those who are developing Benefit-Risk methodologies for assessing medicines that there are four key stages: Framing the decision; Identifying the benefits and risks; Assessing the benefits and risks; and Interpretation and recommendation. Underpinning these was an overarching eight-step framework:

1. Decision context;
2. Building the Value Tree;
3. Value Tree refinement;
4. Assessing relative importance;
5. Evaluating options;
6. Evaluating uncertainty;
7. Concise presentation of results – visualisation;
8. Final recommendation.

All the methodologies currently being developed by pharmaceutical companies and regulators incorporate these steps, whether explicitly or implicitly. This overarching framework provides the basis for common ground and agreement on the principles for benefit-risk assessment.

There are, however, two particularly challenging issues within the conduct of a benefit-risk assessment, one being the assessment of relative importance, and the other, the valuation of the options (medicine under investigation, comparator(s), placebo). With respect to determining relative importance, there is limited consensus with regard to the methods to be applied, and a perception that the process is highly complicated. With regard to valuing the options, there is a range of opinion as to whether valuation should be qualitative, semi-quantitative, or quantitative.

This meeting will bring together experts from the pharmaceutical industry to debate and discuss the two critical issues of relative importance (weighting) and valuing the option, with regard to the utilisation of these within a Benefit-Risk framework. We will discuss these from the perspective of their utilisation by a regulatory authority. It is planned that the discussion will be facilitated around real case studies.

Objectives

- Provide a forum for industry to review methods and discuss the utilisation of weighting and values in making the benefit-risk decision.
- Provide industry perspectives of which weighting and value standards could be used by regulatory agencies, and what issues need to be addressed within the benefit risk-framework.
- Provide the opportunity to put forward proposals for discussion at the CIRS annual benefit-risk meeting, scheduled for June 2013, on the principles that should be applied in these steps of the framework

Venue

The Workshop will take place at the Sofitel Hotel, Philadelphia, USA, on Thursday 13 December 2012, commencing at 08.30 and finishing at 18.00.

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Thursday 13th December 2012

SESSION 1: BENEFIT-RISK ASSESSMENTS: RELATIVE IMPORTANCE FOR BENEFIT-RISK ASSESSMENT - WHAT ARE THE METHODOLOGIES?		
08:30	**Chairman's Introduction**	**Prof Stuart Walker,** Founder, CIRS
08:40	**Principles, rationale and types of methodologies for assessing relative importance**	**Dr Bennett Levitan,** Director, Epidemiology, Janssen Research & Development, USA
09:20	**Discussion**	
	Case Studies of different methodologies for relative importance:	
09:30	**A ranking/qualitative approach**	**Dr Elias Kouchiakji,** Executive Medical Director & **Dr Qi Jiang,** Executive Director, Global Biostatistical Science, Amgen, USA
10:00	**The swing weighting approach**	**Dr James Felli**, Research Fellow, Eli Lilly, USA
10:30	**The IMI Tysabri evaluation**	**Dr Diana Hughes,** Vice President, Worldwide Safety Strategy, Primary Care Business Unit Lead Pfizer Inc, USA
11:00	**Break**	
11:30	**Round Table Discussion: What are the potential methodologies for regulatory assessment?**	
12:15	**Development of recommendations to regulators**	
12:45	**Chairman's Summary**	
13:00	**Lunch**	

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SESSION 2: BENEFIT-RISK ASSESSMENTS: VALUING THE OPTIONS FOR BENEFIT-RISK ASSESSMENT - WHAT ARE THE APPROACHES?		
14.00	**Valuing the Options**	**Prof Stuart Walker,** Founder, CIRS
	Focus on Valuing the Options in a Benefit-Risk Framework	
14.05	**A Company Case Study**	**Dr Marilyn Metcalf,** Director, Benefit Risk Analysis USA GlaxoSmithKline, USA
14.35	**A Company Case Study**	**Dr Carmen Bozic,** Senior Vice President and Global Head, Drug Safety and Benefit-Risk Management Biogen Idec, USA
15.05	**Break**	
15.35	**Round Table Discussion: Quantitative, Qualitative, or Hybrid?**	
16.45	**Should relative importance and valuing be combined in making the benefit-risk assessment for company submission or agencies review?**	**Dr Becky Noel,** Senior Research Scientist, Eli Lilly, USA
17.30	**Chairman's Summary**	
18.00	**Close of meeting**	