The Patient's Role in the Benefit-Risk Assessment for the Submission and Review of New Medicines

25-26 April 2012

PROGRAMME

Tylney Hall Hotel, Hampshire, UK

CENTRE FOR INNOVATION IN REGULATORY SCIENCE
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Organiser
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3 April 2012

Centre for Innovation in Regulatory Science Workshop

Background

The Patient's Role in the Benefit-Risk Assessment for the Submission and Review of New Medicines

As the framework for benefit-risk is developed it has become apparent that the role the patient should play in informing the regulatory decision is increasingly important. However it also seen as an area that is complex in terms of eliciting a perspective in terms of benefit and harms which is based on evidence generation from patients being studied during the clinical development as well as how the patient's perspective should be used in regulatory decision-making.

Indeed at the last CIRS Benefit-Risk workshop all of the syndicate groups made a recommendation that going forward it is important to include the patient's perspective early in the framing process as well as taking these into the weighing process when evaluating conditions involving subjective benefits and harms. It is believed by agencies and companies that patient input could be invaluable in informing the thinking of decision- makers such as regulators and researchers.

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 groups are now undertaking pilot projects to try and apply the models/methodologies to real world cases. The question that is being asked is how and when patients should be involved in informing the benefit-risk decision.

This workshop will explore these issues by gaining a perspective from various stakeholders in the development and review of new medicines with a particular emphasis on the opportunities and barriers to including patients' perspectives in the submission and review of new medicines. This workshop will be a precursor to provide a proposal for discussion at the CIRS annual Benefit-Risk Workshop in June 2012 where the question being posed is; when and how should patients be involved and what would facilitate their involvement with regard to the benefit-risk assessment of new medicines?

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Workshop Objectives

- **Identify the issues and opportunities** for patients, companies and regulators in including patient's views in the benefit-risk assessment of new medicines**.**
- **Clarify how as well as when** patient's views should be incorporated into the benefit-risk assessment of new medicines?
- **Development of a Proposal for discussion at the CIRS June workshop, Identifying the** methodologies and how to achieve a consensus on a scientifically accepted approach for including patients' perspectives in benefit-risk decisions

Venue

The Workshop will take place at the Tylney Hall Hotel, Hook, Hampshire, UK commencing at 13.00 on 25 April and finishing at 13.00 with lunch on 26 April 2012.

Style and Participation

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

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Day 1: 25 April 2012

13:00 Registration & Lunch

SESSION 1: DEVELOPING THE CASE FOR THE INCLUSION OF PATIENTS' PERSPECTIVES IN A FRAMEWORK FOR BENEFIT-RISK ASSESSMENT		
14.00	**Chairman's welcome and introduction**	**Prof Sir Alasdair Breckenridge,** Chairman, Medicines and Healthcare products Regulatory Agency, UK
	What are the opportunities and issues to including patients' perspectives on benefits and risks of new medicines for utilisation in the decision to submit and decision to approve/reject *This session will outline the individual perspectives on the importance of having patients' input into both the development and approval of new medicines – outlining the why as well as the issues and hurdles that each stakeholder has in ensuring that the patient view is considered and understood.*	
14.10	**Patient Viewpoint**	**Dr Mary Baker,** President, European Brain Council
14:30	**Payer viewpoint**	**Prof Bruno Flamion,** Professor of Pharmacology, University of Namur, Belgium
14:50	**Industry viewpoint**	**Moira Daniels,** Vice President, Regulatory, Policy, Intelligence and Labelling, AstraZeneca, UK
15:10	**Discussion**	
15.20	**Break**	
	What are the current Initiatives/models being used for new medicines, and how and when are patients' perspectives being included in the decision making process? *There are already various models and activities that are undertaken to provide patient perspective during the development, review and reimbursement decision of a new medicine. This session to outline how these models work using examples from how different organisations collect and utilise patients' perspectives*	
15:50	***EMA approach***	**Prof Hans-Georg Eichler**, Senior Medical officer, European Medicines Agency
16.15	***US FDA Approach***	**Dr Janice Soreth,** Deputy Director, US Food and Drug Administration Europe Office
16.40	***Company Case Study***	**Dr James Cross,** Regulatory Program Director, Genentech Inc., USA
17:00	***NICE Model for inclusion of Patients***	**Victoria Thomas,** Associate Director: Patient and Public Involvement Programme, National Institute for Health and Clinical Excellence, UK
17.20	**Discussion**	

SESSION 2: SYNDICATE SESSIONS	
17.30	**Introduction to Syndicate Groups**
17.40	**Syndicate Discussions** **Syndicate A: How should patients be involved in the benefit-risk decision and why?** **Chair: Dr Mary Baker,** President, European Brain Council **Rapporteur: TBC** **Syndicate B: When should patients be involved and what are the possible methodologies for the decision to Submit** **Chair: Dr Diana Hughes,** VP Worldwide Safety Strategy Primary Care, Pfizer **Rapporteur: Dr Susan Welsh** VP, MSA TA Head, Oncology & Immunology, Bristol-Myers Squibb **Syndicate C: When should patients be involved and what are the possible methodologies for the decision to Approve?** **Chair: Prof Robert Peterson**, Executive Director, Drug Safety and Effective Network, Canadian Institutes of Health Research **Rapporteur: TBC**
18.30	**Close of day one**
19.00	**Reception**
19.30	**Dinner**

Day 2: 26 April 2012

SESSION 2: SYNDICATE SESSIONS CONTINUE	
08.30	**Syndicate sessions resumes** **Syndicate A: How should patients be involved in the benefit-risk decision and why?** **Chair: Dr Mary Baker,** President, European Brain Council **Rapporteur: TBC** **Syndicate B: When should patients be involved and what are the possible methodologies for the decision to Submit?** **Chair: Dr Diana Hughes,** VP Worldwide Safety Strategy Primary Care, Pfizer **Rapporteur: Dr Susan Welsh** VP, MSA TA Head, Oncology & Immunology, Bristol-Myers Squibb **Syndicate C: When should patients be involved and what are the possible methodologies for the decision to Approve?** **Chair: Prof Robert Peterson**, Executive Director, Drug Safety and Effective Network, Canadian Institutes of Health Research **Rapporteur: TBC**
10.30	**Close of Syndicate Discussions and Coffee**

SESSION 3: INCLUSION OF PATIENTS IN BENEFIT-RISK DECISION-MAKING FOR SUBMISSION AND REVIEW: HOW AND WHEN?		
11.00	**Chairman Introduction**	**Prof Robert Peterson,** Executive Director, Drug Safety and Effective Network, Canadian Institutes of Health Research
11.05	**Feedback from Syndicate Sessions**	
11.45	**Panel Discussion** *This session is to have a reaction from different stakeholders to the ideas suggested by the syndicates as well as to facilitate discussion.*	
	Payer Viewpoint	**Prof Angela Timoney**, Chair, Scottish Medicines Consortium
	Views from patient groups	**Jean Mossman,** Policy Lead, European Federation of Neurological Associations, UK
	Industry Perspective	**Dr David Jefferys,** Senior Vice President, Global Regulatory, Government Relations, Public Affairs and European Product Safety Eisai Europe Ltd, UK
	Regulatory Perspective	**Barbara Sabourin,** Director General, Therapeutic Products Directorate, Health Canada
12:45	**Chairman's Summary – Next Steps**	
13.00	**Close of Workshop**	