The Patient's Voice in Clinical Development:

Can Patients contribute to the Benefit-Risk Assessment of New Medicines?

13 &14 March 2013

PROGRAMME

Woodlands Park Hotel, Cobham, Surrey, UK

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

Background

Patient's Voice in Clinical Development

As companies and agencies currently work on methodologies to develop a framework for making benefit risk decisions and also to communicate to stakeholders, there has been a growing awareness that the most important stakeholder's voice, the patient, is a critical component in making Benefit Risk decisions for new medicines. This is true for both the development phase to ensure companies are developing medicines of value to patients as well as for the regulatory review of new medicines where there may be a difference between what the patient identifies as the maximum acceptable risk and minimum acceptable efficacy to that perceived by the regulatory agency.

In 2012 CIRS held a workshop on "**The Patient's Role in the Benefit-Risk Assessment for the Submission and Review of New Medicines"** and the consensus from this meeting was that patients should be involved in providing information for the benefit risk decision throughout the lifecycle of a medicine including the early and late stages of development and the regulatory review.

This has raised questions in terms of what are the appropriate methodologies for obtaining this information, at what level should this be done, the disease or product, who in the company is responsible for acquiring the information and how do regulators and decision makers view data generated by companies in relation to patients needs. In addition other issues that also need to be discussed are, how to ensure patient advocacy is separate from product advocacy, is this an area in which companies and agencies can collaborate, how to ensure the patients' voice is being heard and do patient advocates represent all patients with a specific disease?

This workshop will explore these issues by providing a perspective from various stakeholders in the development and review of new medicines with a particular emphasis on potential methodologies, the opportunities and barriers to hearing the patients' perspectives with regard to benefits and risk, at both the disease and product level.

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Workshop Objectives

- **Identify key methodologies that are being used to** capture patient's needs in relation to benefits and risks by companies and regulators at both the disease and product level**;**
- **Discuss the potential opportunities for utilization of current and new approaches** as well as the hurdles in both acquiring patient's views and subsequently these being incorporated into the benefit-risk assessment of new medicines
- **Develop of proposals for appropriate patient "voice" pathways in clinical development ,** identifying what methodologies can be used to achieve scientifically acceptable approaches for including patients' perspectives in the construction of the benefit-risk decision.

Venue

The Workshop will take place at the Woodlands Park Hotel, Cobham, Surrey, UK commencing at 09:00 on 13th March and finishing at 13:00 with lunch on 14th March 2013.

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: 13 March 2013

08:30 Registration

SESSION 1: THE INCLUSION OF PATIENTS' PERSPECTIVES IN UNDERSTANDING THEIR NEEDS IN TERMS OF BENEFITS AND RISKS AT THE DISEASE AND PRODUCT LEVEL		
09.00	**Chairman's welcome and introduction**	**Dr Mary Baker,** President, European Brain Council
09:10	**Keynote Presentation – Can Patients Contribute to the Benefit-Risk Assessment of New Medicines?**	**Prof Michel Goldman,** Executive Director, Innovative Medicines Initiative
	How do patients currently inform and companies and agencies identify needs at the disease level in order to influence research and early development? *This session will discuss the different perspectives on what is done currently to acquire or provide information on patients' needs in a disease area and what the potential opportunities that are open to patients to influence the development of new medicines and to meet these needs?*	
09.35	**Industry viewpoint**	**Robin Evers,** Vice President, Worldwide Safety and Regulatory, Pfizer, UK
09.55	**Regulatory viewpoint**	**Dr Franceso Pignatti**, Head of Section Oncology Safety & Efficacy of Medicines, European Medicines Agency
10.15	**Discussion**	
10.30	**Break**	
11.00	**Is a collaborative model for identifying patients' perspective on Benefits and Risks at the disease level a precompetitive area?**	**Dr Frank Rockhold,** Senior Vice President, Global Clinical Safety and Pharmacovigilance, GlaxoSmithKline, USA
11.25	**How useful is it for patient groups to collaborate to ensure that their voice is heard by companies, agencies and payers and what are the opportunities and hurdles?**	**Patricia Pellier,** Vice President Regulatory Affairs EMEA, Celgene, Switzerland
11.50	**Discussion**	
12.00	**Reflection from the Patients perspective** *This session is to have a reflection from the different patient organisations and their viewpoints on the inclusion of Patients perspectives in the development and review of new medicines.* **Jean Mossman,** Policy Lead, European Federation of Neurological Associations, UK **Achim Kautz,** Vice President, European Liver Patients Association **Jeremiah Mwangi,** Policy and External Affairs Director, International Alliance of Patients' Organizations, UK	
12.30	**Lunch**	

Day 1: 13 March 2013

SESSION 2: WHAT ARE THE KEY METHODOLOGIES BEING ADVOCATED AT THE PRODUCT LEVEL TO ENSURE THAT THE PATIENTS' VOICE IS BEING HEARD IN CLINICAL DEVELOPMENT?		
13.30	**Introduction to Session 2**	
	What are the key methodologies being used in clinical development to identify patients' views and what are the pros and cons in how these can inform the Benefit Risk decision by individual stakeholders? Viewpoints on the current methodologies available *This session will discuss the different perspectives on what is being done currently to acquire information on patient's views with regard to medicines in development that will aid companies and regulators.*	
13.35	**Industry viewpoint**	**Dr Jamie Cross,** Program Director, Product Development Regulatory, Genentech Inc, USA
14:00	**Regulatory viewpoint**	**Prof Steffen Thirstrup,** Former Director of Licensing Division, Danish Health and Medicines Authority
14:25	**Academic viewpoint**	**Dr Reed Johnson,** Distinguished Fellow and Principal Economist, Health Preference Assessment Group, Research Triangle Institute, USA
14.55	**Discussion**	
15.00	**Introduction to the syndicates**	**Dr Neil McAuslane,** Director, CIRS
15:15	**Syndicate Discussions** **Syndicate A: What are the Critical Success Factors that will enable the involvement of the Patients' perspectives on Benefits and Harms to contribute to the future success of Research and Development of New Medicines?"** **Chairman: Thomas Lonngren,** Strategy Advisor **Rapporteur: Frederic Ivanow,** Senior Director, Janssen, UK **Syndicate B: What are the Critical Success Factors for the assessment of the Patients' perspectives on Benefits and Harms to contribute to the regulatory review?** **Chairman: Professor Robert Peterson,** Executive Director**,** Drug Safety and Effectiveness Network, Canadian Institutes of Health Research **Rapporteur: Dr Louise Gill,** Senior Director, Global Regulatory Affairs, GlaxoSmithKline, UK	
18.00	**Close of day one**	
19.00	**Reception**	
19.30	**Dinner**	

Day 2: 14 March 2013

SESSION 3: THE PATIENTS VOICE IN DEVELOPMENT: THE RECOMMENDATIONS		
08.30	**Chairman Introduction**	**Prof Sir Alasdair Breckenridge** Former Chairman, MHRA, UK
08.40	**Feedback from Syndicate Sessions**	
09.10	**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 Excellence, UK	**Victoria Thomas,** Associate Director: Patient and Public Involvement Programme, National Institute for Health and Clinical Excellence
	Patient groups Viewpoint	**Jean Mossman,** Policy Lead, European Federation of Neurological Associations, UK
	Industry Viewpoint	**Dr Isabelle Stoeckert,** Head, Global Regulatory Affairs Europe/Canada, Bayer Pharma AG, Germany
	Regulatory Viewpoint	**Prof Steffen Thirstrup,** Former Director of Licensing Division, Danish Health and Medicines Authority
10.00	**Break**	
	The utilisation of social media and new technology to gain a better understanding of patients' needs: How could it be appropriately harnessed for clinical development?	
10:45	**Industry viewpoint**	**Moira Daniels,** Vice President, Regulatory, Policy, Intelligence and Labelling, AstraZeneca, UK
11.05	**Agencies viewpoint**	**Dr John Skerritt,** National Manager Therapeutic Goods Administration, Australia
11:25	**Patient viewpoint**	**Achim Kautz,** Vice President, European Liver Patients Association
11.45	**Discussion**	
11.50	**Chairman's Summary – Next Steps**	
12.00	**Close of Workshop**	