TOPRA

THE ORGANISATION FOR PROFESSIONALS IN REGULATORY AFFAIRS

Agenzia Italiana del Farmaco

AIFA

ANNUAL SYMPOSIUM 2011

Regulatory Science and market access to medicines in Europe

Thursday 13th to Friday 14th October 2011

Crowne Plaza Hotel, Rome, Italy

Organised in conjunction with

AIFA, Agenzia Italiana del Farmaco, Rome, Italy

Working Party
Paolo Biffignandi – VI.REL Pharma, Italy (Chair) Silvia Fabiani, AIFA, Italy Pietro Folino Gallo, AIFA, Italy Gabriella Conti, AIFA, Italy Vera Franzen – Regulatory Affairs Consultant, Sweden Ana Gaspar – Pharmaffairs Consultants, Portugal Alan Hunter – Consultant, UK Tony Humphreys – EMA Margareth Jorvid – LSM Group, Sweden Brenton E James – EU Consultant, UK Patrizia Nestby – PAREXEL, Consulting, The Netherlands Axel Wenzel – Pharma Scientific Services Team, Germany

	Thursday 13th October 2011
08:00	**Registration and Exhibition**
09.00	**Welcome to 2011 Symposium**
	Lynda Wight - *Executive Director*, *TOPRA* Paolo Biffignandi - *Chair of TOPRA Symposium Working Party and Director* VI.REL Pharma S.a.s., Italy Guido Rasi – *Director General, Agenzia Italiana del Farmaco (AIFA), Italy and Executive Director-Elect of the European Medicines Agency (EMA)* Martin Seychell - *Deputy Director General for Consumers and Health, DG SANCO, European Commission*

SESSION 1:
Shifting focus from marketing authorisation to patient access

Leader: Patrizia Nestby, Senior Consultant, PAREXEL Consulting, The Netherlands

This session will provide an update on the strategic initiatives aiming at improving the collaboration and sharing of data between European regulatory agencies and bodies for Health Technology Assessment (HTA). The overall aim of these initiatives is to reduce duplication of work to the extent possible and to make valuable medicinal products available to the patient in the shortest possible timeframe. In line with this, pharmaceutical companies are increasingly trying to integrate the requirements from regulators as well as from HTA bodies in their development programmes. Experiences gained by regulators, HTA bodies and industry in this area will be presented. Ultimately, these initiatives are expected to benefit innovation and the development of medicinal products in therapeutic areas with a clear medical need.

Chairs:
Guido Rasi - *Director General, Agenzia Italiana del Farmaco (AIFA), Italy and Executive Director-Elect of the European Medicines Agency (EMA)*
and
Patrizia Nestby – *Senior Consultant, PAREXEL Consulting, The Netherlands*

09.30	**Introduction –** Patrizia Nestby – *Senior Consultant, PAREXEL Consulting, The Netherlands*
09:35	**Regulator's view – arranged marriage or living apart together?**
	Aginus Kalis - *Executive Director Medicines Evaluation Board, The Netherlands, and Chair of Heads of Medicines Agencies Management Group (HMA-MG)*
	• Heads of Medicines Agencies (HMA) Strategy 2011 – 2015: where do we meet Health Technology Assessment (HTA)? • Current initiatives and developments – joint Scientific Advice • Efficacy and effectiveness - a world apart? • Does culture medicine matter? • Vision for the future
09:55	**HTA/payer's view – Joint Scientific Advice (SA): next steps**
	Ad Schuurman - *Head of the Business Contact Centre and International Affairs at the Dutch Health Care Insurance Board (CVZ), Netherlands and President of Medicine Evaluation Committee (MEDEV)*
	• A mini crash-course: what is regulatory assessment, Health

	Technology Assessment (HTA) and pricing/reimbursement (differences/overlap)? • EUnetHTA guideline development for guiding Health Technology Assessment (HTA) (comparator, endpoints, internal/external validity) • Current initiatives and development – joint Scientific Advice, pilot multi-stakeholder Scientific Advice • Vision for the future
10:15	**Industry view – Single, parallel or joint stake holder consultation in early and late stage drug development**
	Mats Marfalt - *European Regulatory Affairs and Director CV/GI Portfolio, AstraZeneca, Sweden*
	• Experiences from pilot multi-stakeholder Scientific Advice at EMA • Reflection on how multi-stakeholder Scientific Advice could be improved • Vision for the future
10:35	**Panel discussion with session speakers and invited panellists:** Paolo Siviero **-** *Head of Economic Strategy and Pharmaceutical Policy, Italian Medicines Agency (AIFA)* Spiros Vamvakas - *Head of Section, Scientific Advice, Human Medicines Special Areas, European Medicines Agency*
	The speakers will take questions from the floor – this is your opportunity to explore the topic more fully.
11.00	**Break**

Thursday 13th October 2011

SESSION 2:
HTA, Pricing & Reimbursement and Market Access in Europe

Leader: Alan Hunter, Past President of TOPRA and Consultant, UK

Following on from Session 1 we now introduce the hurdle of pricing into the equation for market access.

The first part of this session deals with the differing institutions and procedures used in several Member States to obtain marketing authorisations, health technology assessments and pricing and reimbursement. The situation in Italy, the UK and Portugal will be reviewed.

In the second part of the session, we take a look at pricing policies in Europe and how the revision of the Pricing Transparency Directive may be able to help in addressing equity in access and sustainability of Healthcare Systems. There will be a brief update on the progress of the European Commission consultation process for possible revision of the Transparency Directive.

A panel discussion then follows with the session speakers.

Chair:
Alan Hunter – *Past President of TOPRA and Consultant, UK*

11:30	**Introduction : Alan Hunter, Past President of TOPRA and Consultant, UK**
	Institutions and Procedures reviewing Pricing and Reimbursement, HTAs and Regulatory Approval
11:35	**Italy** Agency viewpoint
	Pietro Folino Gallo - *Director, Office of OsMed and HTA activities, AIFA, Italy*
	• Combining licensing, pricing, reimbursement and governance of pharmaceutical expenditure in a single Agency • How to conciliate innovation and budget constraints • Managed Entry Agreements: an innovative way for facilitating access to new medicines
11:50	**UK** Industrial viewpoint
	Richard Barker - *Author on the future of healthcare and former Director General of UK Association British Pharmaceutical Industry (ABPI), UK*
	• In the UK three agencies deal with market authorisation (MHRA), HTA (NICE) and pricing and reimbursement (Department of Health, DoH). • For HTA there are 4 different UK nations to deal with and in England the assessing body is The National Institute of Clinical Evidence(NICE) • Pricing is dealt with by the UK Department of Health (DoH) under the Pharmaceutical Price Regulation Scheme (PPRS) and changes are being proposed to develop Value Based Pricing from 2014.The benefits and drawbacks of such changes will also be addressed
12:05	**Portugal** Agency viewpoint
	Isaura Vieira - *Director of economic evaluation and market monitoring department, Infarmed, Portugal*
	• View from Infarmed in Portugal where the regulatory agency deals with reimbursement and HTA in addition to marketing authorisation, but pricing involves the Ministry of Economics.
12.20	**The European Commission proposals for updating the Transparency Directive**: An update from the European Commission relayed by Alan Hunter

12:25	**Pricing policies in Europe: will a revision of the Transparency Directive be able to address equity in access and sustainability of healthcare systems?**
	Andrea Rappagliosi - *Vice President European Government Affairs, GlaxoSmithKline, Belgium*
	• Impact of the financial crisis on pricing policies and patients access to medicines; • Single market and international reference pricing: a hurdle or a booster? • Entry managed schemes: the new frontier of improved access performances and system efficiency.
12:45	**Panel discussion with this session's speakers:**
	The speakers will take questions from the floor – this is your opportunity to explore the topic more fully.
13.00	**Lunch**

Thursday 13th October 2011

SESSION 3
Combination Products, drug/device

Leader: Margareth Jorvid, LSM Group, Sweden

This session will cover the different ways for a drug/device combination to reach the market. It will explore the different regulatory procedures and different actors involved and their roles. It will also explain the different legislation for drugs and devices and how to classify a drug/device combination.

Chairs:
Margareth Jorvid - *Senior Partner of LSM Group, Sweden*
and
David Jefferys - *Eisai, UK and TOPRA President*

14:00	**Introduction**
14:05	**Differences in Drug and Device terminology, authorities and approval processes to market**
	Margareth Jorvid - *Senior Partner of LSM Group, Sweden*
	• What pharma regulatory professionals need to know about devices • What device QA/RA need to know about medicinal products
14:25	**Drug/devices regulated as medicinal products**
	Ann O'Connor - *Director of Human Products Authorisation and Registration, Irish Medicines Board (IMB), Ireland*
	• Current approach when a medicine has a device aspect • Links to Notified Bodies • New developments e.g. ATPs, Personalised medicines and IVDs • Proposals/questions
14:45	**Drug/devices regulated as devices**
	Giovanni Di Rienzo – *Technical Expert, BSI Healthcare, UK*
	• The Notified Body role • Dossier preparation and content • The process to CE marking
15:05	**Panel discussion with this session's speakers** and Gabriella Conti - *Head of European Assessment Office, AIFA, Italy*
	The speakers will take questions from the floor – this is your opportunity to explore the topic more fully.
15.30	Break

Thursday 13th October 2011

SESSION 4:
Pharmacovigilance: The New European Picture

Leader: Paolo Biffignandi, VI.REL Pharma, Italy

This session will cover the regulatory changes stemming from Regulation no. (EU) No 1235/2010 and Directive 2010/84/EU: how Pharmacovigilance is changing across the EU. What will be the impact of the new Committee? Can Pharmacovigilance be considered as a tool to assess effectiveness of a medicinal product rather than to be a reporting activity only? The EU Commission made an effort: are National Agencies ready to give feedback to healthcare professionals and patients? In essence, do we consider Pharmacovigilance a regulatory burden or a gain for the European healthcare scenario?

Chairs:
Aginus A.W.Kalis - *Executive Director, Medicines Evaluation Board (MEB), The Netherlands, and Chair of the Heads of Medicines Agencies Management Group (HMA-MG)*
and
Martin Seychell - *Deputy Director General for Consumers and Health, DG SANCO, European Commission*

16:00	**Introduction from the chairs**
16:05	**The new Pharmacovigilance legislation**
	Sabine Straus - *Head of MEB Pharmacovigilance Department and member of EMA PhVWP, The Netherlands*
	•
16:30	**Pharmacovigilance: burden or gain?**
	June Raine - *Director of Vigilance and Risk Management of Medicines, MHRA, UK*
	•
16:50	**How Pharmacovigilance will influence effectiveness of a product**
	Peter Arlett - *Head of Sector for Pharmacovigilance and Risk Management at the EMA*
	•
17:10	**Panel discussion with this session's speakers plus invited panellists:** Fernanda Ferrazin- *Head Pharmacovigilance Department at AIFA, Italy* Daniela Melchiorri - *CHMP member and member of the Eudravigilance Steering Committee*
	The speakers will take questions from the floor – this is your opportunity to explore the topic more fully.
17.30	**Break**

Thursday 13th October 2011

Introduction to the Italian competent authorities

In Italy the various aspects of medicines and devices are managed between three key authorities: The Ministry of Health, Istituto Superiore di Sanita (ISS) and Agenzia Italiana del Farmaco (AIFA). In this session we will hear about the specific roles of these three authorities and how they work together.

17:45 - 18:30

Moderator:

Paolo Biffignandi, TOPRA Past-President

Panel:

Ferruccio Fazio - *Italian Ministry of Health*

Medicines, Medical Device and Health: the role of the Italian Ministry of Health

Enrico Garaci - *President, Istituto Superiore di Sanità, Italy*

Medicines and Research: the role of ISS

Sergio Pecorelli - *President of AIFA, Italy*

AIFA and the Challenges and Opportunities of Globalisation

Guido Rasi - *Director General, Agenzia Italiana del Farmaco (AIFA), Italy and Executive Director-Elect of European Medicines Agency*

AIFA in the European Network

	Networking Reception

Friday 14th October 2011

SESSION 5 : Clinical Trials

Leader: Axel Wenzel, Managing Director, Pharma Scientific Services Team Ltd P.SS.T, Germany

Many clinical trials in specific patient populations need to be conducted on a multinational level in various countries. Within the European Union (EU), these multinational clinical trials require approval of the same clinical trial application by the national competent authorities as well as the national ethics committees. This bureaucratic process might not only be time-consuming but also result in varying country-specific final decisions requiring repeated adaption of clinical trial documents. This session will discuss the current regulatory situation in EU for multinational clinical trials as well as attempts to make the CTA administration easier.

Chairs:

Axel Wenzel - *Managing Director, Pharma Scientific Services Team (P.SS.T), Germany*
and
Carlo Tomino - *Coordinator of the Pre-Authorisation Department, AIFA and Italian CTFG representative, Italy*

09:00	**Introduction**
09:05	**Revision of the Clinical Trials Directive 2001/20/EC**
	Stefan Fuehring – *Administrator, Pharmaceuticals unit D3, DG SANCO, European Commission*
	The European Commission is planning to put forward in 2012, a legislative proposal to revise the Clinical Trials Directive 2001/20/EC.1 To assess the impact of this revision, a public consultation was held from 9 October 2009 to 8 January 2010 (the '2009/10 public consultation'). The responses, together with a summary of them, have been published on the 'clinical trials website' of 'Health and Consumers' Directorate-General (DG SANCO).
09:25	**The Voluntary Harmonization Procedure (VHP): does it work in practice?**
	Heiko E. von der Leyen – *CEO, Hannover Clinical Trial Center GmbH, Germany*
	In order to harmonize the conduct of multinational clinical trials within the EU, the Clinical Trials Facilitation Group (CTFG) has set up a novel procedure for approval of multinational clinical trials - the Voluntary Harmonization Procedure (VHP). VHP enables a coordinated assessment of the clinical trial application (CTA) between the concerned national competent authorities and comprises three phases. With a positive VHP opinion, national approval of a study will be obtained within 10 days. This new procedure will be discussed in detail with respect to clinical study management.
09:45	**Agency viewpoint**
	Chantal Bélorgey - *Chef du Département de l'Evaluation des Essais Cliniques et des Médicaments à Statut particulier/ Head Department of Evaluation of Clinical trials and Special Status Medicinal Products, Chair CTFG* *Agence Française de Sécurité Sanitaire des Produits de Santé (Afssaps), France*
	•
10:05	**Panel discussion with this session's speakers and invited panellists:** Luca Pani, *Italian Alternate Permanent Member, Committee for Human Medicine Products (CHMP), Member, Scientific Advice Working Party (SA-WP) and Working Party on Central Nervous System (WP-CNS), EMA, Member of the Committee for Clinical Neurosciences, AIFA, Italy* Patrizia Popoli, *Research Director at the Department of Medicines, Commission for Phase I Clinical Trials, Istituto Superiore di Sanità (ISS), Italy*
	Harmonisation of clinical trial legislation National competent authorities (NCA) and Ethics committee (Institutional review Boards (EC / IRB) play a decisive role in the complexity of the clinical study

	approval process. Worldwide, these are national processes, but multicentre clinical trials are often running internationally. Whereas in EU there are now attempts to simplify the approval process by NCA (see Voluntary Harmonisation Procedure), the processes and the requirements of ECs are even not yet harmonised on a national level. The need for harmonisation of clinical trial authorisation requirements and processes will be discussed.
	The speakers will take questions from the floor. This is your opportunity to explore the topic more fully.
10.30	**Break**

Friday 14th October 2011

SESSION 6:
Paediatric Clinical Trials

Leaders: Ana Gaspar, Pharmaffairs Consultants, Portugal and Vera Franzen, Consultant, Sweden

This session will cover:

- *Ethical, scientific and regulatory challenges of paediatric clinical trials*
- *Need for PIPs and Class Waivers*
- *PK/PD and scientific advice*
- *Adequate dosage forms*
- *Child-friendly communications*

Chairs:
Paolo Rossi - *member of the EMA Paediatric Committee PDCO, and Professor of Paediatrics , University of Rome "Tor Vergata", Rome Italy*
and
Paolo Biffignandi – *Director, VI.REL Pharma S.a.s, Italy*

11:00	**Introduction**
11:05	**The Role and Challenges of the PDCO in defining paediatric clinical trials**
	Paolo Rossi – *member of the EMA Paediatric Committee PDCO, and Professor of Paediatrics , University of Rome "Tor Vergata", Rome, Italy*
	• Waiver, compliance, etc
11:25	**Access to Market for Paediatric Medicines: is it working?**
	Agnes Saint Raymond – *Head of Human Medicines Special Areas, EMA*
	•
11:45	**Industry view: Integrating a PIP into a development programme**
	Axel Breitstadt - *Head of European Regulatory Affairs, Merck Sharp & Dohme (Europe) Inc, Belgium*
	•
12:05	**Panel discussion with this session's speakers plus invited panellist :** Adriana Ceci *(Enpr-EMA)*
	The speakers will take questions from the floor – this is your opportunity to explore the topic more fully.
12.30	**Lunch**

Friday 14th October 2011

SESSION 7:
Decentralised and Mutual Recognition procedures

Leader: Brenton James, EU Consultant, UK

This session will present the successes of these two important procedures in the year 2011 to date. Figures will be presented on the number and type of medicinal products that have been registered via these procedures, together with information on referrals to CMD (The Co-ordination Group for Mutual Recognition and Decentralised Procedures) and arbitrations to CHMP (The Committee for Medicinal Products for Human Use). The key role that Italy plays in these procedures will be discussed and with ideas on the development of the CMD.

Generic products can be approved via DCP and the Centralised Procedure; information and direction of the Heads of Agencies Strategy towards the registration of these products will be shared.

Chairs:
Brenton James - *EU Consultant, UK*
and
Laura Braghiroli – *Office for Evaluation and Registration, MRP and DCP Procedures, AIFA, Italy*

13:30	**Introduction: Brenton James – EU Consultant, UK**
13:35	**CMD statistics and successes in 2011**
	Peter Bachmann - *Senior Expert, Department of European and International Affairs, Federal Institute for Drugs and Medical Devices (BfArM), Germany*
	•
13:55	**National Competent Authorities (NCA) role in CMD(h) – the Italian experience**
	Sandra Petraglia - *Agenzia Italiana del Farmaco (AIFA), Italy and Italian CMDh member*
	•
14:05	**Generic Products. What's the optimal Procedure?**
	Marcus Muellner – *CEO, Austrian Medicines and Medical Devices Agency,AGES* , Austria
	• Generic applications via Mutual Recognition, Decentralised and the Centralised Procedure • Challenges and solutions
14:25	**Panel discussion with this session's speakers**
	The speakers will take questions from the floor – this is your opportunity to explore the topic more fully.
14.50	**Break**

Friday 14th October 2011

SESSION 8:
Consumer Health

This session will cover some of the special challenges facing the consumer health industry. These challenges will be illustrated by a case study on the use of the MRP-DCP procedures and the Italian non-prescription market. This will be contrasted by the situation when using the centralised procedure and how this differs to the MRP-DCP route. Finally the future challenges for the self-care sector will be reviewed in the light of new legislation such as pharmacovigilance, falsified medicines, variations etc .

Chairs:
Cheryl Hall - *Regulatory Affairs Director, Boots Pharmaceuticals, UK*
And
Fernanda Ferrazin- *Head Pharmacovigilance Department at AIFA, Italy*

15:20	**Introduction**
15:25	**Case study**
	Gianfranco Giuliani – *Director of Regulatory Affairs, Angelini, Italy*
	• MRP-DCP: experience in working with AIFA, lessons learnt and regulatory tips • Visibility of non-prescription medicines on the Italian market: website, advertising, pack design, distribution • Positive initiatives to encourage self-care in Italy
15:45	**Centralised procedure for non-prescription medicines - let's review the situation**
	Agnes Saint Raymond – *Head of Human Medicines Special Areas, EMA,*
	• Overview of the procedure including eligibility aspects, scientific advice, pre-submission meetings, etc. • Naming and packaging for non-prescription medicines • Experience so far at central level - useful tips for applicants
16:05	**Current and future challenges for the self-care sector**
	Christelle Anquez-Traxler - *Regulatory and Scientific Affairs Manager, AESGP, Belgium*
	• Pharma package: Smart implementation for non-prescription medicines - Pharmacovigilance – aspects of most relevance for non-prescription medicines & implementation - Falsified medicine legislation • Other aspects (variations, fees, pack design, procedure, etc)
16:25	**Panel discussion with this session's speakers plus** Gabriella Conti - *Head of European Assessment Office, AIFA, Italy*
	The speakers will take questions from the floor – this is your opportunity to explore the topic more fully.
16.50	**End of session**
16:50	**Closing -** Guido Rasi, *Director General, Agenzia Italiana del Farmaco (AIFA), Italy and Executive Director-Designate of European Medicines Agency (EMA*)),
16:55	**Close of Symposium -** David Jefferys, *TOPRA President 2011*

17:00	**End of Symposium**