ANNUAL SYMPOSIUM 2014

13 – 15 October 2014
The Square, Brussels

Organised in conjunction with
FAMHP/FAGG/AFMPS
(Belgium's regulatory agency)

Matching modern regulation with modern medicine – a patient-centred approach to regulatory affairs?

TOPRA acknowledges the support of the Belgian Regulatory Affairs Society (BRAS) in the development of this meeting.

Working Party	FAMHP
Margareth Jorvid – *LSM Group, Sweden (co-chair)* Christine Mayer-Nicolai – *Merck KGaA, Germany* Sarah Montagne – *Bayer HealthCare, UK* Anne De Bock – *AstraZeneca, Belgium* Joao Da Silva Duarte – *H. Lundbeck A/S, France* Kuo-Wei CHAN – *President BRAS***,** *BRAS representative* Alan Hunter – *Consultant, UK* **Plus for medical devices:** Hilde Viroux – *Alcon, Belgium* Ruth Foster – *Terumobct, Belgium* **Advisers to programme:** June Raine – *Chair PRAC, MHRA, UK* Tomas Salmonson – *Chair CHMP, MPA, Sweden* Peter Bachmann – *Chair CMD(h), BfArM, Germany* David Jefferys – *Eisai, UK* **From parallel symposia:** **Veterinary medicines** – Ray Harding, *Independent Consultant, UK* **Medical devices** – Margareth Jorvid – *LSM Group* **And support from TOPRA SPIN groups and TOPRA IN groups:** Cecil Nick – *Parexel and TOPRA Biotech SPIN*	Xavier De Cuyper – *CEO, FAMHP* Greet Musch – *FAMHP Coördinator, Director General, DG PRE authorisation* Vanessa Binamé – *Director General, DG POST Authorisation and FAMHP contact point for Medical Devices Symposium* Josiane Van Der Elst – *Director General, DG Inspection* Pascal Giloteau – *Coördinator Supportive Services and FAMHP contact point for Budget and Logistics* Dries Minne – *Head of Veterinary Division at DG, PRE Authorisation and FAMHP contact point for Veterinary Medicines Symposium* Els Geeraerts – *Coördinator International Relations Services AG* Ann Verhoye – *Staff member, DG PRE Authorisation* Philippe De Buck – *Head of division authorisations at DG Inspection* Augustin Coppee – *Management Support Services AG* Ann Eeckhout – *Head of division Communication Sevices AG, FAMHP* Daniel Brasseur – *CHMP member* Bart Van der Schueren – *Alternate CHMP member, UZ Leuven/KU Leuven*

	Monday 13 October 2014
11.30-12.30	**Registration, Exhibition and Lunch**
12.30	**Welcome to 2014 Symposium**
	Lynda Wight – *Executive Director*, *TOPRA* Xavier De Cuyper – *CEO, **F**ederal **A**gency for **M**edicines and **H**ealth **P**roducts (FAMHP), Belgium*
12.40 **Keynote speech:** Nicola Bedlington – *Executive Director, European Patient Forum (EPF), Belgium*	
SESSION 1: The new Clinical Trial Regulation **Leader:** Anne De Bock - *AstraZeneca, Belgium*	
The newly adopted Clinical Trial Regulation has been put in place to improve access to clinical research in the EU. With the European Commission discussing key aspects of the Regulation, EU member states presenting insights into their local implementation plans, the European Medicines Agency's (EMA) assessment on the functionality of the future portal and a lively panel discussion including ethics committee participation – this session will provide an in-depth overview of translating the Regulation into practice.	
Chair: Kristof Bonnarens – *Head of Division R&D, FAMHP, Belgium*	
13.00	**Introduction**
13.05	**Key aspects of the new Clinical Trial Regulation and next steps**
	Stefano Soro – *Head of Unit, Medicinal products – quality, safety and efficacy, Health and Consumers Directorate-General, European Commission*
	• Key aspects from a procedural viewpoint • Proposed system • Implementation steps
13.25	**Implementation of the Clinical Trial Regulation from a member state perspective**
	Martyn Ward – *Chair Clinical Trials Facilitation Group (CTFG), and Clinical Trials Unit, MHRA*
	• Operational aspects of the procedure • How ethics committees will be involved • Challenges from an assessor's viewpoint
13.45	**The Portal – functional assessment**
	Fergus Sweeney - *Head of Inspections and Human Medicines Pharmacovigilance, EMA*
13.55	**Panel discussion,** moderated by Anne De Bock – *AstraZeneca, Belgium*
	The speakers and Greet Musch – *Director General DG PRE authorisation, FAMHP, Belgium,* plus panel contributors from the floor representing ethics committees , patient organisations (Anne Vergison, *Mutualités Socialistes, Belgium*), and industry (Nick Sykes, *Pfizer, UK*) will take questions from the floor – this is your opportunity to explore the topic more fully.
14.40	**Break**

Monday 13th October 2014	
SESSION 2: The FAMHP with focus on the patient- early phase development and unmet medical needs **Leader:** Greet Musch - *Director general DG PRE authorisation, FAMHP, Belgium* *Presented with the support of BRAS* 	
The FAMHP's new business plan, with a central focus on the patient, will be highlighted. This session will give a deeper insight into the way the Centre of Excellence, Early Phase Development (EPD), is developing in Belgium and how it is aligned within the European regulatory network. *The new legislative framework on early access, where there is close cooperation between the National Competent Authority (FAMHP) and the Reïmbursement Authority (RIZIV-INAMI), will be presented and illustrated by an industry case (Belgian Regulatory Affairs Society, BRAS).*	
Chair: Professor Jean-Paul Degaute – *President of Scientific Commission, FAMHP, Belgium*	
15.25	**The FAMHP serving the patient: Vision 2014–2020**
	Xavier De Cuyper – *CEO, FAMHP, Belgium*
15.40	**Early phase development: Strengthening the clinical research environment in Belgium and Europe**
	Walter Janssens – *Coördinator Centre of Excellence EPD, FAMHP, Belgium*
	• Newest moving fields of interest (inclusion of patients in early phase clinical trials, estimation of risk for reproductive effects?, combination of products a real need?, biomarkers for activity and adverse effect) • Critical points of attention at EU level (safety pharmacology studies: principle of 3R's, need for GLP compliance and non-GLP screening process: how to integrate? multinational studies for rare diseases, what is needed for support of studies with ATMP's in early phases) • Businessplan for the upcoming 3 years: highlights
16.05	**Unmet medical needs: New legislation FAMHP-RIZIV/INAMI**
	Greet Musch – *FAMHP, Belgium* and Ri De Ridder–*, RIZIV-INAMI, Belgium*
	• New national legislation on early temporary authorisation and early temporary reimbursement for unmet medical needs: demand driven versus supply driven ? • Justification of "unmet medical need" and assessment of Benefit-Risk • Link with European initiatives : adaptive licensing – MOCA
16.15	**Unmet medical needs: Early dialogue – An industry perspective**
	Mimi De Ruyck - *Bayer Healthcare , Market access, Belgium*
	• Opportunities & challenges of an early dialogue between pharma and FAMHP-RIZIV/INAMI • Recent scientific regulatory technical advice experiences from a local pharma perspective • Early access to patients with an unmet medical need: the way forward!
16.35	**Panel discussion** moderated by Ingrid Theeuwes – *Belgian Regulatory Affairs Society (BRAS), Belgium*
	The speakers, plus panel contributors from the floor representing a patient organisation (Heidi Goethals, *Christelijke Mutualiteiten, Belgium)*, political representatives, the King Baudouin Foundation (Tinne Vandesande) and Health, Science & Technology (HST) Group (Dr Delaporte) will take questions from the floor – this is your opportunity to explore the topic more fully.
16.55	**Short comfort break**

Monday 13th October 2014	
SESSION 3: The FAMHP with focus on the patient from two perspectives: falsified medicines and vaccines **Leader:** Greet Musch – *Director General, DG PRE authorisation, FAMHP, Belgium* *Presented with the support of BRAS*	

• The Falsified Medicines Directive: The FAMHP viewpoint
• Centre of Excellence Vaccines through the regulatory pathway:
  ○ Scientific advice – Rapporteurships – Pharmacovigilance Committee (PRAC) experiences – Critical good manufacturing practice (GMP) issues (FAMHP/WIV)
  ○ Industry cases (BRAS)	

Chair: Greet Musch – *Director General, DG PRE authorisation, FAMHP, Belgium*	
17.25	**The Falsified Medicines Directive: The FAMHP perspective**
	Josiane Van Der Elst – *FAMHP, Belgium*
	The Falsified Medicines Directive: what's in it for the patient? • Increased control on the product • Increased control on the operators in the supply chain • Increased control on internet sales
17.40	**Questions from the floor**
17.45	**Centre of Excellence Vaccines throughout the regulatory pathway: Experiences gained and challenges identified by authorities**
	Pieter Neels - *Consultant, Belgium* and Nele Berthels – *FAMHP, Belgium*
	• Vaccines in the pipeline: (new) vaccines for "new" populations. • Vaccines in the licensing procedure: role as Rapporteur to establish a benefit-risk evaluation Vaccines on the market: a continued effort to evaluate the efficacy/effectiveness and the safety of the vaccine
18.10	**Centre of Excellence Vaccines throughout the regulatory pathway: Experiences gained and challenges identified by industry**
	Michel Stoffel – *GlaxoSmithKline Biologicals , Belgium*
	• Why is vaccine development so complex? • What are the regulatory challenges faced by vaccine industry within and outside the EU? • Will vaccine innovation remain sustainable in the future?
18.30	**Panel discussion,** moderated by Kuo Wei Chang*, BRAS President, Belgium* plus invited contributors Professor Marc Van Ranst and Yves VanLaethem, Belgium
	The speakers will take questions from the floor – this is your opportunity to explore the topic more fully.
18.55	Networking drinks reception in the Exhibition Hall
20.00	Close of Day 1 and freedom to explore Brussels

Tuesday 14 October 2014	
Introduction to Day 2 – TOPRA	
SESSION 4: Paediatric development **Leader:** Margareth Jorvid – *LSM Group, Sweden*	
The Paediatric Regulation came into force in 2007. The Paediatric Committee (PDCO) was established, responsible for coordinating the EMA's work on medicines for children. The Committee's main role is to determine the studies that companies must carry out in children as part of paediatric investigation plans (PIPs).This session will discuss experiences to date from the EMA and the PDCO. A concept paper was released by the European Commission at the end of 2013 for public consultation with a view to updating the guideline on the format and content of applications for PIPs/waivers/deferrals, taking into account the experiences gained with the paediatric legislation. The session will discuss proposals for future development and what data will be needed for the Commission's 10-year final review report.	
Chair: Daniel Brasseur – *CHMP member, FAMHP, Belgium*	
08.45	**Introduction**
08.50	**Challenges with filing and managing a successful PIP**
	Margareth Jorvid – *LSM Group, Sweden*
09.00	**The EMA's role in paediatric medicines**
	Paolo Tomasi - *Head of Section - Paediatric Medicines, European Medicines Agency*
	• Experiences/statistics • PIPs and modifications • Full withdrawals and waivers
09.20	**The work of PDCO – contributing to better medicines for children**
	Dirk Mentzer – *Chair PDCO, and Paul-Ehrlich-Institut, Germany*
	• PDCO's work and experience • Condition *vs* indication • Partial compliance
09.40	**Future changes following the 2013 public consultation**
	Florian Schmidt – *Unit D5, European Commission*
	• Update of guideline/public consultation • Future development • What data will be needed for the draft final report
10.00	**Panel discussion,** moderated by *Margareth Jorvid – LSM Group, Sweden*
	The speakers, plus invited guest Koenraad Norga – *Vice Chair, PDCO, and member FAMHP, University Hospital, Antwerpen, Belgium,* will take questions from the floor – this is your opportunity to explore the topic more fully.
10.30	**Break**

Tuesday 14th October 2014	
SESSION 5: Adaptive licensing **Leader:** FAMHP	
This session will explore European views on adaptive licensing, the UK early access scheme and how industry is planning for this shift in regulatory development design. An extended panel will discuss the wider implications for an adaptive approach, with the views of the European Commission, patients, prescribers and health technology assessment bodies (HTAs) being aired.	
Chair: Professor Minne Casteels – *SAWP member, FAMHP, KU Leuven, Belgium.*	
11.10	**Introduction**
11.15	**European view on adaptive licensing**
	Tomas Salmonson – *Chair, CHMP, and Medical Products Agency (MPA), Sweden*
	• Adaptive licensing from an EU perspective • EU pilot
11.35	**The UK national view in a European context**
	Robert Hemmings – *Chair of Scientific Advice Working Party (SAWP), and Unit Manager, Statistics and Pharmacokinetics Unit, MHRA, UK*
	• UK pilot of the Early Access to Medicines Scheme (EAMS)
11.55	**GlaxoSmithKline (GSK) approaches to adaptive licensing**
	James Anderson – *Director, European Partnerships, Government Affairs, Public Policy & Patient Advocacy*,*GSK*, *UK*
	• What industry sees as the benefit to this paradigm shift • Approaches in EU and similar systems in US and other markets • GSK experiences
12.15	**Panel discussion** moderated by Professor Minne Casteels with Olga Solomon, *Unit D5, European Commission*, Francesca Cerreta, *EMA* and Frank Hulstaert–KCE, *Belgium HTA* plus panel contributor from the floor Marc Dooms, *Vlaams Patiëntenplatform, Belgium*
	The speakers will take questions from the floor – this is your opportunity to explore the topic more fully.
12.40	**Lunch**

Tuesday 14th October 2014	
SESSION 6: HTA and regulatory development **Leader:** Joao Da Silva Duarte – *H. Lundbeck A/S, France*	
This session will cover how best to integrate regulatory strategy with health technology assessment (HTA) planning during drug development. In a time when such integration is crucial for market access, challenges remain in the evidence needed to be generated to fulfil both regulatory and HTA needs and how parallel advice can add value to such development. Some of the latest initiatives in Europe to facilitate such alignment will be discussed, such as the shaping of early dialogues and experience derived from the EU HTA Network (EUnetHTA).	
Chair: João da Silva Duarte – *H. Lundbeck A/S, France*	
13.40	**Introduction:** João da Silva Duarte – *Regulatory Intelligence & Policy Manager, H. Lundbeck A/S, France*
13:45	**Parallel HTA scientific advice: what is the experience so far?**
	Francesca Cerreta - *Senior Scientific Administrator, European Medicines Agency*
	• Regulators' perspective on parallel HTA scientific advice; • Learning outcomes from past experiences; • Current and future challenges and opportunities for this tool.
14:05	**HTA Trends: How can EU dialogues contribute to better drug development?**
	Francois Meyer - *Advisor to the President, International Affairs, Haute Autorité de santé (HAS), France*
	• HTA body perspective on current HTA challenges in EU; • Current initiatives in the field (e.g. SEED consortium) and HAS experience; • Current and future challenges and opportunities for HTA bodies.
14:25	**Integrating HTA and regulatory strategy: how to generate value?**
	Iman Barilero – *Vice President, Regulatory Development Strategy & Policy, Lundbeck, Denmark*
	• Industry's perspective on how to generate evidence and value in drug development; • Integration of HTA considerations in early and late regulatory strategy; • Current and future challenges and opportunities for drug development in EU.
14:45	**Panel discussion** moderated by Joao Da Silva Duarte
	With invited panellist Tapani Piha , *Head of e-Health and Health Technology Assessment Unit, DG SANCO, European Commission, EU.* The speakers will take questions from the floor – this is your opportunity to explore the topic more fully.
15:20	**Break**

Tuesday 14th October 2014	
SESSION 7: Personalised medicines **Leader:** Christine Mayer-Nicolai – *Merck KGaA, Germany*	
Personalised medicines offer tremendous promise to provide focused treatments for targeted patient populations. However, manufacturers are challenged with developing more complex targeted clinical trials, which may offer clear evidence of a drug's efficacy in a specific patient subgroup based on the mode of action of the drug and factoring in the development of a suitable companion diagnostic at an early point in time. The parallel development of drug and diagnostic poses significant challenges to the development and market adoption of a personalised medicine. This session will focus on exploring questions related to how regulators are designing evidentiary standards for personalised medicines versus how manufacturers are working to meet these standards, and the challenges involved in making personalised medicines a reality for overall patient care. *The term personalised medicines is used synonymously with stratified medicines in this context, following the approach taken in the report published by the European Commission at the end of 2013.*	
Chair: *Genevieve Michaux – Counsel, Hunton & Williams LLP, Belgium*	
16.00	**Introduction**
16.05	**Challenges in bringing a personalised medicine to market**
	Alan Morrison – *Vice President Regulatory Affairs, Amgen, UK*
	How to: • Develop a completely specified genomic classifier of the patients likely to benefit from a new drug • Establish analytical validity of the classifier • Use the specified classifier to design and analyse a new clinical trial to evaluate effectiveness
16.25	**Questions and answers discussed in scientific advice for personalised medicines**
	Professor Dieter Deforce – *SAWP member, FAMHP, and University of Gent, Belgium*
	• The most frequently asked questions • The most important potential pitfalls in development • How to address changing scientific knowledge in implementing personalised approaches for marketed drugs
16.45	**Panel discussion moderated by** Christine Mayer-Nicolai – *Merck KGaA, Germany* with invited panellist Agnès Mathieu*, Unit D5, European Commission* and panel contributor from the floor Professor Jacques Degrève*, Belgian Medical Society of Oncologists*
	The speakers will take questions from the floor – this is your opportunity to explore the topic more fully.
17.30	**TOPRA Board update and student poster winners announced**
18.00 –	**Networking drinks in exhibition hall followed at 19.00 by buffet dinner in the Panoramic Hall**

Wednesday 15 October 2014	
Introduction to day 3 – TOPRA	
SESSION 8: Transparency **Leader:** Alan Hunter – *Consultant, UK*	
Openness and transparency are enshrined in the Treaty of Europe and contribute to democracy and good administration. The EMA sees transparency as a key consideration in delivering its services to patients and society.To this end, the EMA has developed a policy on access to documents and more recently a draft policy on publication and access to clinical trial (CT) data. In this session, the EMA will detail these policies, including how the draft policy will be fine-tuned and implemented *Transparency is a two-way process and industry expects transparency in agency decision-making and procedures. The European industry association, EFPIA, will give its views on how the EMA draft policy on access to CT data may be improved while safeguarding patient privacy, respecting the integrity of regulatory systems and ensuring sufficient investment in biomedical R&D.* *Central to this discussion is the patient and we shall have a European patient representative to explain what the patient really wants in terms of transparency.* *Following these talks there will be a panel discussion also involving a guest representative from research academia.*	
Chair: Alan Hunter – *Consultant, UK*	
09.00	**Introduction**
09.05	**Transparency - A key consideration for the EMA**
	Noël Wathion – *Head of Stakeholders and Communication Division (ad interim)*, European Medicines Agency
	• Importance of transparency • Policy on access to documentation • Draft policy on access to CT data • Fine-tuning the draft policy to meet the needs of all stakeholders
09.25	**The EFPIA perspective on transparency**
	Richard Bergstrom – *Director General, European Federation of Pharmaceutical Industries and Associations (EFPIA)*
	• Requirements for transparency in regulatory agency decision-making • Measures to improve the EMA draft policy on CT data access • Maintaining safeguards for patient privacy • The importance of commercial confidentiality • Respecting the integrity of the regulatory process • Maintaining incentives to invest in biomedical research
09.45	**The patient's perspective - fact and fiction**
	Marco Greco – *President, EFCCA and PRAC Patient Representative Alternate*
	• How does data disclosure support patients? • Patient's expectations as clinical trial participants
10.00	**Panel discussion** moderated by Alan Hunter**,** with invited guest representative for academic research Professor Katelijne De Nys – *KU Leuven , Belgium* and panel contributor for the floor Martine Vanhecke, *Test-aankoop, Belgium*
	The speakers will take questions from the floor – this is your opportunity to explore the topic more fully.
10.30	**Break**

Wednesday 15th October 2014	
SESSION 9: Pharmacovigilance **Leader:** Sarah Montagne – *Bayer HealthCare, UK*	
This session will provide an engaging insight into the first two years following the implementation of the EU pharmacovigilance (PV) legislation and will aim to assess how well the legislation has met its high-level objectives of reducing the burden of side-effects and optimising the use of medicines in the EU. *How far have we come in engaging patients and healthcare professionals in the system? How risk-proportionate is the EU system? To what extent does greater transparency relate to better information for patients? These are just some of the questions that come to mind... and yet more questions remain: What does the patient see? Is all the hard work of implementation making a difference? What has worked well for regulators and industry alike... and what has worked less well?* *This session will reflect equally on the hopes for the future and the priorities moving forward, as patients and healthcare professionals become more engaged and as all those involved in the EU system seek to provide better information on medicines.*	
Chair: Sarah Montagne – *Bayer HealthCare, UK*	
11.10	**Introduction**
11.15	**Pharmacovigilance – A view from PRAC and a national competent authority (NCA)**
	Almath Spooner – *Co-Chair Pharmacovigilance Risk Assessment Committee (PRAC), and Health Products Regulatory Authority(HPRA), Ireland*
	• To reflect on the impact of the PV legislation since its introduction and to assess current status with regard to meeting stated objectives • What went well for PRAC and what needs further development? • What is the role of the national competent authorities supporting PRAC? • How will PRAC further develop the engagement of patients and healthcare professionals in the system, and what challenges remain?
11.35	**The impact on industry**
	Judith Weigel – *Pharmacovigilance/ Pharmaceutical Guidelines, vfa (German association of Research-Based Pharmaceutical Companies), Germany*
	• To reflect on the impact of the PV legislation on industry since its introduction • What are the challenges, and what can be done to address them? • What are the hopes for the future, as industry seeks to meet regulatory requirements and works to provide better information on medicines?
11.55	**Patient perspective**
	Marco Greco – *President, EFCCA and PRAC Patient Representative Alternate*
	• How does a patient input into PRAC discussions? • How can patients engage more widely in ongoing procedures? • What do patients see, and what do they want to see?
12.15	**Panel discussion** moderated by Sarah Montagne**,** with invited PRAC representative, Jean-Michel Dogne*, Belgium* and Noël Wathion*, EMA* The speakers will take questions from the floor – this is your opportunity to explore the topic more fully.
12.50	**The close of 2014 and a look forward to 2015**
13.00	**Close of symposium by TOPRA President**

Tuesday 14th October 2014	
PARALLEL SESSION: Biosimilars **Leader:** Cecil Nick – *Parexel, UK*	
This session will describe the challenges facing the introduction of biosimilars – their interchangeability, switching, extrapolation of indications and the guidelines available.	
Chair: Professor Paul Declerck – *Vice Chair, Commission for Medicines for Human Use FAMHP Belgium, KU Leuven.*	
08.45	**Introduction**
08.50	**EMA pioneering guidelines revisited**
	Karen De Smet –*Senior Non-clinical Evaluator, FAMHP, Belgium*
09.10	**Interchangeability and switching**
	Cecil Nick – *Vice President (Technical), PAREXEL International, UK*
09.30	**Extrapolation of indications**
	Janne Komi - *alternate CHMP member, Senior Medical Officer , Finnish Medicines Agency (Fimea), Finland*
09.50	**Panel discussion moderated by Professor Paul Declerck** With invited panellist Joris Van Assche, *Febelgen Belgium*.
10.30	The speakers will take questions from the floor plus representation of prescribers , BSMO – this is your opportunity to explore the topic more fully. Close of session