The EFGCP Children’s Medicines Working Party 3rd Annual Conference

EU Paediatric Regulation: First European Experiences & Strategic Outlook

8-9 October 2007 - Management Centre Europe, Brussels, Belgium

organised by the

Children’s Medicines Working Party

European Forum for Good Clinical Practice

‘where science & ethics meet’
Conference Rationale and Objectives

The EU paediatric regulation was published in the EU Official Journal December 2006 and is in force since January 2007. The EMEA Paediatric Committee (PdCo) will be established by 26th July 2007. Ten years after the first US paediatric legislation the EU is now the second major region with a legislation aimed at strengthening paediatric pharmaceutical research to improve our own children's health as well as to offer better armamentariums to the global paediatric clinical community. Canada has introduced an own paediatric legislation in 2006, and more countries and regions are considering comparable moves. The highly successful US paediatric legislations are in the process of re-authorisation which is expected to be done well before their sunset end of September 2007.

The EFGCP is a forum that fosters strategic dialogue across the barriers of affiliations that are involved in clinical research. The Children's Medicines Working Party is composed of academic paediatricians, regulators, patients' advocacy groups, and pharmaceutical industry associates. This is the third annual conference after the first two successful conferences 2005 and 2006. This year’s conference will focus on first experiences with the EU Paediatric Regulation and will combine this debate with a strategic lookout towards the next years of global paediatric research.

As this topic has now intensively been discussed on numerous conferences in both Europe and the USA and many more conferences are currently offered on this topic, the EFGCP Children's Medicines Working Party 3rd Annual Conference will make a special effort to avoid repetitive standard presentations that professionals involved into the field of paediatric drug development have already listened to repeated times. We will start the conference with 3 high level presentations on first hands-on experiences with the EU paediatric regulations from EMEA, PdCo, and pharmaceutical industry, and will in the afternoon have 3 additional update keynote presentations on the USA, Japan, and paediatric study design. As the past two conferences, the main focus will be the lively discussion in the breakout sessions and the conclusions from these breakout sessions that will be discussed in the afternoon plenary.

To facilitate newcomers’ full participation at this high level conference, this year EFGCP offers an additional ½ day pre-conference workshop in the afternoon of Monday October 8 that will provide fundamental background knowledge on off-label use of drugs in children, US and EU paediatric legislations, the basics of paediatric clinical trials and of the general challenges how to integrate paediatric drug development into the general drug development process.

Again, this conference is designed to be as interactive as possible. Number and length of key presentations will be limited to allow as much discussion in the breakout sessions and in the plenary as possible. Conference participation will allow direct interaction with the key players in this initiative, will allow access to first-hand information, and will be a first opportunity to contribute with detailed examples how the EU Paediatric Regulation will be interpreted by the various stakeholders. It will help participants from the pharmaceutical industry to understand the scope and consequences of the EU Paediatric Regulation and its interaction with the US paediatric legislations. It will help the participating regulators to understand concerns and planning of the pharmaceutical industry. It will help the participating academic researchers to get a feeling for how this regulation will contribute to strengthen European Paediatric Research.

The next EFGCP Children's Medicines Working Party Annual Conference will take place in October 2008.
Conference Participants
The conference audience will include representatives and participants from regulatory authorities, academic paediatric research, health professionals, pharmaceutical & biotech companies, and patient and parents’ organisations.

Conference Language
The language of the conference is English.

Conference Dinner
A working dinner will be organised the evening prior the workshop (Monday 8 October, 2007)

Faculty
Daniel Brasseur  University of Brussels; Federal Agency for Medicines and Health Products, Belgium; Paediatric Committee (PdCo), EMEA
Elisabeth Carter  Pfizer, USA
Mark Del Monte  American Academy of Pediatrics, USA
Fergal Donnelly  DG Research, European Commission
Mats Ericson  Wyeth Research
Tricia Fowler  GlaxoSmithKline, United Kingdom
Sabine Fuerst-Recktenwald  Sanofi-Aventis, Germany
Petra Knupfer  Baden-Württemberg Ethics Committee, Germany
Pirjo Laitinen-Parkkonen  PdCo & National Agency for Medicines, Finland
Birka Lehmann  Federal Institute for Drugs and Medical Devices (BfArM), Germany
Samuel Maldonado  Johnson & Johnson, PhRMA Pediatric Group, USA
Dirk Matthys  University Hospital of Gent, Belgium
Hidefumi Nakamura  National Centre for Child Health & Development, Japan
Pietro Panei  Italian National Institute of Health, Italy
José Ramet  European Academy of Paediatrics (EAP-CESP), Universitair Ziekenhuis Antwerpen & ZNA Koningin Paola Kinderziekenhuis, Antwerp, Belgium
Klaus Rose  EFGCP Children’s Medicines Working Party; Roche, Switzerland
Nicola Ruperto  IRCCS Istituto G. Gaslini, Italy
Agnes Saint-Raymond  Scientific Advice and Orphan Drugs, European Medicines Agency (EMEA)
Tsveta Schyns  European Network for Research on Alternating Hemiplegia (ENRAH), EURORDIS & European Genetic Alliances' Network (EGAN), Austria
Monika Seibert-Grafe  Coordination Centre for Clinical Trials (KKS) & PAED-Net (German Paediatric Clinical Trials Network), Germany
Nathalie Seigneuret  
Human Unit-Safety & Efficacy of Medicine, European Medicines Agency (EMEA)

Hannesjörg W. Seyberth  
Professor Emeritus, University of Marburg; European Society for Developmental Perinatal & Paediatric Pharmacology (ESDP); European Network for Drug Investigation in Children (ENDIC), Germany

Rosalind Smyth  
Medicines for Children Research Network (MCRN), United Kingdom

Hans Stötter  
SwissMedic, Switzerland

Melissa Tassinari  
Pfizer, USA

Frank Wells  
European Forum for Good Clinical Practice (EFGCP), United Kingdom

Lothar-Bernd Zimmerhackl  
University of Innsbruck, Austria
Agenda

Monday, 8 October 2007

14.00-18.00

Pre-Conference Workshop

Background:
Off-label Use of Drugs in Children
US & EU Paediatric Legislations
Basics of Child Physiology
Basics of Paediatric Clinical Trials

Faculty:

Tricia Fowler
Director of Special Projects, Clinical Pharmacology Discovery Medicine, GlaxoSmithKline, United Kingdom

Klaus Rose
Chairman, Children's Medicines Working Party, European forum for Good Clinical Practice (EFGCP); Head, Paediatrics, Roche, Switzerland

Nathalie Seigneuret
Scientific Administrator, Human Unit-Safety & Efficacy of Medicine, European Medicines Agency (EMEA)

Hannesjörg W. Seyberth
Professor Emeritus, University of Marburg; European Society for Developmental Perinatal & Paediatric Pharmacology (ESDP); European Network for Drug Investigation in Children (ENDIC), Germany

Hans Stötter
Clinical Reviewer, SwissMedic, Switzerland

19.30: Working Dinner for all the participants of the Children's Medicines Working Party Annual Conference
Tuesday, 9 October 2007

08:00  Registration and Welcome Coffee
08:45  Welcome

*Klaus Rose*, Chairman, Children's Medicines Working Party, European Forum for Good Clinical Practice (EFGCP); Head, Paediatrics, Roche, Switzerland

**Session 1**

*First Experiences*

09:00  PdCo

*Daniel Brasseur*, Professor of Paediatric Medicine, University of Brussels & Federal Agency for Medicines and Health Products, Belgium; Member, Paediatric Committee (PdCo), European Medicines Agency (EMEA)

09:20  EMEA

*Agnes Saint-Raymond*, Head of Sector, Scientific Advice and Orphan Drugs, European Medicines Agency (EMEA)

09:40  Industry

*Elisabeth Carter*, Associate Director, Regulatory Affairs, Pfizer, USA

*Klaus Rose*, Chairman, Children's Medicines Working Party, European Forum for Good Clinical Practice (EFGCP); Head, Paediatrics, Roche, Switzerland

10:00  Coffee Break

10:30  Breakout Session I: ‘Focus on Specific Issues’

**Working Group 1**:
‘First Experiences EMEA’

*Chair: Birka Lehmann*, Federal Institute for Drugs and Medical Devices (BfArM), Germany

*Rapporteur: Tsveta Schyns*, Secretary General, Vienna Office, European Network for Research on Alternating Hemiplegia (ENRAH), EURORDIS & European Genetic Alliances’ Network (EGAN), Austria

**Working Group 2**:
‘First Experiences Industry’

*Chair: Pietro Panei*, Senior Researcher, Drug Research and Evaluation Department, Italian National Institute of Health, Italy

*Rapporteur: Melissa Tassinari*, Senior Director, Worldwide Regulatory Affairs & Quality Assurance, Pfizer, USA

**Working Group 3**:
‘First Experiences Paediatric Research Networks’

*Chairs: Rosalind Smyth*, Director, Medicines for Children Research Network (MCRN), United Kingdom & *Monika Seibert-Grafe*, Head, Coordination Centre for Clinical Trials (KKS) & PAED-Net (German Paediatric Clinical Trials Network), Germany

*Rapporteur: Fergal Donnelly*, Scientific Officer, DG Research, European Commission

12:00  Lunch
13:00 Report from Working Groups & Plenary Debate  
**Chair:** Frank Wells, Consultant, Co-Chairman, Ethics Working Party, European Forum for Good Clinical Practice (EFGCP), United Kingdom

### Session 2

#### Strategic Thoughts on Global Paediatric Research

14:00 American Academy of Pediatrics  
*Mark Del Monte*, Assistant Director, Department of Federal Affairs, American Academy of Pediatrics, USA

14:15 Pharmaceutical Industry  
*Samuel Maldonado*, Johnson & Johnson, PhRMA Pediatric Group, USA

14:30 Stimulation of Paediatric Research in Japan  
*Hidefumi Nakamura*, Director, Division of Clinical Research, National Centre for Child Health & Development, Japan

14:45 **Breakout Session II:**

**Working Group 4:**
‘European Paediatric Research Networks, Training of Clinical Investigators, and Disease-specific Research Networks: Strengths, Gaps, Challenges'  
**Chair:** Nicola Ruperto, Medical Doctor, Pediatria II, PRINTO, IRCCS Istituto G. Gaslini, Italy  
**Rapporteur:** Sabine Fuerst-Recktenwald, Clinical Research Director, Clinical & Exploratory Pharmacology Department, Sanofi-Aventis, Germany

**Working Group 5:**
‘Paediatric Clinical Trial Design'  
**Chair:** Hans Stötter, Clinical Reviewer, SwissMedic, Switzerland  
**Rapporteur:** Hannesjörg W. Seyberth, Professor Emeritus, University of Marburg; European Society for Developmental Perinatal & Paediatric Pharmacology (ESDP); European Network for Drug Investigation in Children (ENDIC), Germany

**Working Group 6:**
‘EU Clinical Trials Directive and Paediatric Clinical Research'  
**Chair:** Lothar-Bernd Zimmerhackl, Director, Department for Kinder und Jugenheilkunde, University of Innsbruck, Austria  
**Rapporteur:** Petra Knupfer, Baden-Württemberg Ethics Committee, Germany

15:50 Coffee Break

16:00 Report from Working Groups & Plenary Debate  
**Chairs:** José Ramet, Chairman, Department of Paediatrics, Universitair Ziekenhuis Antwerpen & ZNA Koningin Paola Kinderziekenhuis, Antwerp, Belgium; European Academy of Paediatrics (EAP-CESP) & Monika Seibert-Grafe, Head, Coordination Centre for Clinical Trials (KKS) & PAED-Net (German Paediatric Clinical Trials Network), Germany

17:00 Conclusions & End of Meeting