

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

*EU Paediatric Regulation: First European
Experiences & Strategic Outlook*

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 groups and the conclusions from these breakout groups 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 working groups 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 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)

Faculty

Peter Arlett	Pharmaceuticals Unit, DG Enterprise and Industry, European Commission
Fergal Donnelly	DG Research, European Commission
Sabine Fuerst-Recktenwald	Sanofi-Aventis, Germany
Mats Ericson	Wyeth Research
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
Ysbrand Poortman	European Genetic Alliances' Network (EGAN), The Netherlands
José Ramet	European Academy of Paediatrics (EAP – CESP) & Koningin Paola Kinderziekenhuis, Belgium
Klaus Rose	EFGCP Children's Medicines Working Party; Roche, Switzerland
Nicola Ruperto	IRCCS Istituto G. Gaslini, Italy
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
Lothar-Bernd Zimmerhackl	University of Innsbruck, Austria

Agenda

Monday, 8 October 2007

Pre-Conference Workshop

Background:

Off-label use of drugs in children, US & EU paediatric legislations,
Basics of child physiology and 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
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 European Experiences

09:00 PdCo

Speaker invited

09:20

EMEA

To be determined, European Medicines Agency (EMA)

09:40

Industry

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

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

10:20

Coffee Break

10:30

Breakout Session I : 'Focus on Specific Issues'

Working Group 1 :

'First Experiences EMEA / PdCo'

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

Rapporteur: *to be determined*

Working Group 2 :

'First Experiences Industry'

Chair: *Hans Stötter, Clinical Reviewer, SwissMedic, Switzerland*

Rapporteur: *Pirjo Laitinen-Parkkonen, Senior Medical Officer, Marketing Authorisation Department, National Agency for Medicines & PdCo, Finland*

Working Group 3 :

'First experiences paediatric research networks'

Introduction: *Rosalind Smyth, Director, Medicines for Children Research Network (MCRN), United Kingdom*

Rapporteur: *To be determined*

12:00

Lunch

13:00

Report from Working Groups & Plenary Debate

Session 2

Strategic Thoughts on Paediatric Clinical Research

- 13:45 USA
Melissa Tassinari, Senior Director, Worldwide Regulatory Affairs & Quality Assurance, Pfizer, USA
- 14:05 Paediatric Study Design
Speaker invited
- 14:25 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 :**
'General European Paediatric Research networks, Treaining 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: *To be determined*
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 Direction and Paediatric Clinical Research'
Chair: *To be determined*
Rapporteur: *Lothar-Bernd Zimmerhackl, University of Innsbruck, Austria*
- 16:05 Coffee Break
- 16:30 Report from Working Groups
- 17:05 Plenary Debate
- 17:50 End of Meeting