

EFGCP Children's Medicines Working Party 6th Annual Conference &  
DIA Paediatric SIAC 4th Forum

# Current & Future Perspectives for Paediatric Medicines: Visions, Daily Challenges, Ways Forward

28 & 29 September 2010  
Canary Wharf, London, United Kingdom

*organised by the*



European Forum for Good Clinical Practice

&



Drug Information Association

[www.efgcp.be](http://www.efgcp.be) – [www.diahome.org](http://www.diahome.org)

## Conference Rationale

The EFGCP Children's Medicines Working Party and DIA Europe are pleased to announce their second joint paediatric conference. Traditional paediatric meetings of both societies in the past and the joint meeting in 2009 have attracted top level speakers from the European Medicines Agency (EMA), Food and Drug Administration (FDA), national authorities, World Health Organization (WHO), academia, pharmaceutical industries and parents & patients' organizations. This second joint program will again offer the opportunity for intensive discussion among stakeholders in different topics relevant for paediatric medicines. We will address visions, daily challenges and ways forward in paediatric drug development. On this basis, the conference will also offer excellent networking opportunities for all attendees.

The conference will include three parallel breakout sessions on each day for a lively interactive discussion. Furthermore, it will provide participants with the opportunity of asking questions by e-mail before the conference and during the morning of day 1, which will be in the afternoon addressed by a speaker panel chaired by Paolo Tomasi, EMA.

## Conference objectives

- Update participants how to deal with paediatric regulatory requirements, scientific and operational challenges
- Exchange experiences with regulatory authorities, academia and industry
- Discuss visions, daily challenges and potential ways to move forward and further improve processes for paediatric drug development

## Programme Committee

Gesine Bejeuhr	Pediatric Special Interest Area Community (Ped SIAC), DIA & vfa Research-Based Pharmaceutical Companies, Germany
Daniel Basseur	PDCO, European Medicines Agency (EMA) & Federal Agency for Medicines and Health Products (FAMHP), Belgium
Vincent Grek	Only For Children Pharmaceuticals (O4CP), France
Agnes Gyurasics	PDCO, European Medicines Agency (EMA) & National Institute of Pharmacy, Hungary
Karl-Heinz Huemer	PDCO, European Medicines Agency (EMA) & Austrian Agency for Health and Food Safety (AGES) PharmMed - Austrian Medicines Agency, Austria
Angelika Joos	Merck Sharp & Dohme, Belgium
Robert "Skip" Nelson	Food and Drug Administration (FDA), USA
Klaus Rose	Children's Medicines Working Party, EFGCP & Granzer Regulatory Consulting & Services, Germany
Sarah Schuppener	Drug Information Association (DIA), Switzerland
Fanny Senez	European Forum for Good Clinical Practice (EFGCP), Belgium
Thomas Severin	Novartis Pharma, Switzerland
Ashley Strougo	Leiden University & Astellas Pharma, The Netherlands
Paolo Tomasi	European Medicines Agency (EMA)
Bernd Zimmerhackl	University of Innsbruck, Austria

## Faculty

Ralph Bax	European Medicines Agency (EMA)
Gesine Bejeuhr	Pediatric Special Interest Area Community (Ped SIAC), DIA & vfa Research-Based Pharmaceutical Companies, Germany
Daniel Brasseur	PDCO, European Medicines Agency (EMA) & Federal Agency for Medicines and Health Products (FAMHP), Belgium
Melanie Carr	European Medicines Agency (EMA)
Susan Conroy	Therakind, United Kingdom
Simon Davies	Teenage Cancer Trust, United Kingdom
Martine Dehlinger-Kremer	European CRO Federation (EUCROF) & Omnicare Clinical Research, Germany
Vincent Grek	Only For Children Pharmaceuticals (O4CP), France
Agnes Gyurasics	PDCO, European Medicines Agency (EMA) & National Institute of Pharmacy, Hungary
Ralf Herold	European Medicines Agency (EMA)
Karl-Heinz Huemer	PDCO, European Medicines Agency (EMA) & Austrian Agency for Health and Food Safety (AGES) PharmMed - Austrian Medicines Agency, Austria
Angelika Joos	Merck Sharp & Dohme, Belgium
Alastair Kent	Genetic Interest Group (GIG), United Kingdom
Dirk Matthys	European Academy of Paediatrics (EAP) & University Hospital Ghent, Belgium
Robert "Skip" Nelson	Food and Drug Administration (FDA), USA
Gesa Pellier	European Federation of Pharmaceutical Industries and Associations (EFPIA) & Novartis Pharma, Switzerland
Representative	Teenage Cancer Trust, United Kingdom (invited)
Klaus Rose	Children's Medicines Working Party, European Forum for Good Clinical Practice (EFGCP) & Granzer Regulatory Consulting & Services, Germany
Renée Simar	INC Research, USA
Ashley Strougo	Leiden University & Astellas Pharma, The Netherlands
Paolo Tomasi	European Medicines Agency (EMA)
Bernd Zimmerhackl	University of Innsbruck, Austria
Martine Zimmerman	Alexion Europe, France

## Conference Language

The language of the Conference will be English.

## Social Event

On the evening of September 28th, all delegates are invited to take part in the Conference social event. Further details will be communicated at a later stage.

# Agenda

Tuesday, 28 September 2010

- 08:00 Registration and Welcome Coffee
- 08:45 **Welcome and Introduction to the Conference**  
*Klaus Rose, Children's Medicines Party, EFGCP & Granzer Regulatory Consulting & Services, Germany*  
*Gesine Bejeuhr, Pediatric Special Interest Area Community (Ped SIAC), DIA & vfa Research-Based Pharmaceutical Companies, Germany*

## Plenary Session 1

### *Visions vs. Daily Operational Challenges*

- Chairperson:** *Klaus Rose, Children's Medicines Party, EFGCP & Granzer Regulatory Consulting & Services, Germany*
- 09:00 **Industry Wish list for Paediatric Drug Development in Europe**  
*Gesa Pellier, European Federation of Pharmaceutical Industries and Associations (EFPIA) & Novartis Pharma, Switzerland*
- 09:30 **Thoughts of the EU Commission on Pan-European Regulatory and Ethical Approval of Clinical Trials in Adults and Children**  
*Speaker invited*
- 10:00 **Strengths and Weaknesses of Academic Structures in European Paediatric Research**  
*Prof. Dirk Matthys, European Academy of Paediatrics (EAP) & University Hospital Ghent, Belgium*
- 10:30 Coffee Break
- 11:00 **Three Parallel Breakout Working Groups, Focus on Specific Issues**
- Group 1:** **Special challenges in neonates**  
Chair: *Robert "Skip" Nelson, Food and Drug Administration (FDA), USA*  
Introduction: *Ralph Bax, European Medicines Agency (EMA)*  
Rapporteur: *(invited)*
- Group 2:** **Cooperation between academia and industry and the way forward in Paediatric Oncology and Rheumatology**  
Chair: *Agnes Gyurasics, PDCO, European Medicines Agency (EMA) & National Institute of Pharmacy, Hungary*  
Introduction: *Bernd Zimmerhackl, University of Innsbruck, Austria*  
Rapporteur: *Ralf Herold, European Medicines Agency (EMA)*

**Group 3:** Operational challenges in paediatric clinical trials and how to overcome them  
Chair: *(invited)*  
Introduction: *Martine Dehlinger-Kremer, European CRO Federation (EUCROF) & Omnicare Clinical Research, Germany*  
Rapporteur: *Renée Simar, INC Research, USA*

12:15 Lunch

## Plenary Session 2

### Getting Paediatric Development Working

13:15 Feedback from Working Groups

Chairperson: *Angelika Joos, Merck Sharp & Dohme, Belgium*

Rapporteur Group 1: *(invited)*

Rapporteur Group 2: *Ralf Herold, European Medicines Agency (EMA)*

Rapporteur Group 3: *Renée Simar, INC Research, USA*

14:15 Report and tentative answers on questions collected pre-conference  
*Paolo Tomasi, European Medicines Agency (EMA)*

15:15 Coffee Break

15:45 Panel discussion and general discussion

Chairperson: *Karl-Heinz Huemer, PDCO, European Medicines Agency (EMA) & Austrian Agency for Health and Food Safety (AGES) PharmMed - Austrian Medicines Agency, Austria*

16:50 Conclusions

*Klaus Rose, Children's Medicines Working Party, EFGCP & Granzer Regulatory Consulting & Services, Germany*

17:00 End of day 1

18:30 Social Event

Keynote Speech on Larger Thinking

*Alastair Kent, Genetic Interest Group (GIG), United Kingdom*

## Wednesday, 29 September 2010

## Plenary Session 3

### The Gap between Technically Doable and Politically Feasible

Chairperson: *Agnes Gyurasics, PDCO, European Medicines Agency (EMA) & National Institute of Pharmacy, Hungary*

09:00 First World Paediatric Research and Global Efforts: How FDA Tries to Boost Paediatric Research for 3<sup>rd</sup> World Diseases?

*Robert "Skip" Nelson, Federal Drug Agency (FDA), USA*

- 09:45      **Funding Paediatric Research: The Spanish Example**  
*Speaker invited*
- 10:15      **Introduction to Breakout Working Groups**  
*Klaus Rose, Children's Medicines Working Party, EFGCP & Granzer Regulatory Consulting & Services, Germany*
- 10:20      Coffee Break
- 10:45      **Three Parallel Breakout Working Groups, Focus on Specific Issues**
- Group 4:      A toolkit for paediatric research for Small and Medium Enterprises (SMEs)**  
    Chair: *Melanie Carr, European Medicines Agency (EMA)*  
    Introduction: *Martine Zimmerman, Alexion Europe, France*  
    Rapporteur: *Susan Conroy, Therakind, United Kingdom*
- Group 5:      Funding for paediatric drug development**  
    Chair: *Vincent Grek, Only For Children Pharmaceuticals (O4CP), France*  
    Introduction: *(invited)*  
    Rapporteur: *(invited)*
- Group 6:      Media, parents & clinical research: challenges & opportunities**  
    Chair: *(invited)*  
    Introduction: *Representative, Teenage Cancer Trust, United Kingdom (invited)*  
    Rapporteur: *Simon Davies, Teenage Cancer Trust, United Kingdom*
- 12:15      Lunch

## Plenary Session 4

### The Way Forward

- 13:15      **Feedback from Working Groups**
- Chairperson: *Daniel Brasseur, PDCO, European Medicines Agency (EMA) & Federal Agency for Medicines and Health Products (FAMHP), Belgium*
- Rapporteur Group 4: *Susan Conroy, Therakind, United Kingdom*  
    Rapporteur Group 5: *(invited)*  
    Rapporteur Group 6: *Simon Davies, Teenage Cancer Trust, United Kingdom*
- 14:15      **Panel discussion and general discussion**
- Chairperson: *Daniel Brasseur, PDCO, European Medicines Agency (EMA) & Federal Agency for Medicines and Health Products (FAMHP), Belgium*
- 15:40      **Conclusions & Farewell**  
*Klaus Rose, Children's Medicines Working Party, EFGCP & Granzer Regulatory Consulting & Services, Germany*  
*Ashley Strougo, Leiden University & Astellas Pharma, The Netherlands*
- 15:50      **End of Meeting**