Joint EFGCP / DIA / EMA Medicines for Children Conference on

Development of Paediatric Medicines: From Learning to Adapting

26 & 27 September 2012
De Vere Venues Canary Wharf, London, United Kingdom

Organised by

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where science & ethics meet

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EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Programme Committee:

Gesine Bejeuhr, VfA (Association of Research-based Pharmaceutical Companies), Germany
Irja Lutsar, PDCO Member for Estonia
Cecile Ollivier, European Medicines Agency
Thorsten Olski, European Medicines Agency
Klaus Rose, Klausrose Consulting, Switzerland
Thomas Severin, Novartis, Switzerland

conferences@efgcp.eu - www.efgcp.eu

Conference Rationale

The EU paediatric regulation is now in force since 2007 and has considerably changed the place of paediatric drug development in the general drug development process. Drug development is no longer possible without considering children. The aim of this conference is to discuss on a high level where we stand with paediatric drug development in Europe, where the partners in drug development, i.e. clinicians, industry, regulators and others, agree and where they disagree, in which area the EU regulation can be considered a success, where the regulation might be modified in the future, where and how its daily interpretation by the EMA will be modified, and where collaboration between the involved players can be improved.

The EFGCP / DIA / EMA Pediatric Conference allows an open discussion between academics, clinicians, regulatory authorities, parents' & patients' associations and pharmaceutical industry about paediatric drug development. The conference is an annual event that goes back to two series of annual conferences: a first one initiated in 2005 by EFGCP, and a second one initiated in 2007 by DIA. Both were organized in tight collaboration with the EMEA, which in 2011 changed its name to EMA. Since 2010 the two conference series have been merged, and since 2011 EMA is officially co-producing the conference as an equal partner.

The EU paediatric regulation has transformed paediatric drug development from a topic discussed by a handful of interested paediatricians, clinical pharmacologists and regulators to an issue that is broadly known within pharmaceutical industry, regulatory authorities, the clinical world and other institutions. More than 1000 PIPs have so far been submitted, and virtually everybody within pharmaceutical industry has heard of the PIP requirement.

The level of detail in the key elements of Paediatric Investigation Plan has been continuously discussed since the Paediatric Regulation came into force. In the 2011 conference, EMA announced that changes will come in this area; this year EMA will announce how these changes will look like.

As in previous conferences, representatives from all stakeholders will give presentations, and on day 1 participants will in three breakout sessions discuss issues in smaller groups. This will allow participants to discuss on eye level and face-to-face with their respective counterparts that otherwise interact predominantly by phone and only occasionally at face-to-face meetings.

Faculty

Gesine Bejeuhr	Pediatric Special Interest Area Community (Ped SIAC), DIA & Vfa Research-Based Pharmaceutical Companies, Germany
Alexander Cvetcovich-Muntanola	INC Research, Spain
Martine Dehlinger-Kremer	Paediatric Working Group European CRO Federation (EUCROF) & Omnicare Clinical Research, Germany
Giorgia Gavriilidou	European Medicines Agency (EMA)
Jordi Llinares	European Medicines Agency (EMA)
Irja Lutsar	PDCO Member for Estonia
Sam Maldonado	Johnson & Johnson, USA
Christoph Male	European Medicines Agency (EMA), Paediatric Committee (PDCO)
Dirk Mentzer	European Medicines Agency (EMA), Paediatric Committee (PDCO)
Genevieve Michaux	Covington & Burling, Belgium
Marek Migdal	Children's Memorial Health Institute & European Network of Paediatric Research at the European Medicines Agency (Enpr-EMA), Poland
Dianne Murphy	Food and Drug Administration (FDA), USA
Cecile Ollivier	European Medicines Agency (EMA)

Thorsten Olski	European Medicines Agency (EMA)
Birgitta Olsson	Swedish Orphan Biovitrum (SOBI), Sweden
Khazal Paradis	Genzyme, The Netherlands
Guido Rasi	European Medicines Agency (EMA)
Bruno Reigner	F. Hoffmann-La Roche, Switzerland
Klaus Rose	Children's Medicines Working Party, European Forum for Good Clinical Practice (EFGCP) & Klausrose Consulting, Switzerland
Agnes Saint-Raymond	European Medicines Agency (EMA)
Tsveta Schyns-Liharska	European Network for Research on Alternating Hemiplegia (ENRAH), Belgium
Thomas Severin	Novartis, Switzerland
Philippa Smit-Marshall	PharmaNet, The Netherlands
Paolo Tomasi	European Medicines Agency (EMA)
Richard Vesely	European Medicines Agency (EMA)
Ulrich Wahn	Pediatric Pneumology & Immunology Dpt, Charité University of Medicine Berlin, Germany
Kerstin Westermark	European Medicines Agency (EMA)

Conference Language

The language of the Conference will be English.

Conference Venue

De Vere Venues Canary Wharf
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1 Westferry Circus, Canary Wharf
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Information & Registration

EFGCP Secretariat: conferences@efgcp.eu / Tel: +32 2 732 87 83 / Skype: efgcpbxl

Agenda

Wednesday 26 September 2012

08:00 Registration and Welcome Coffee

08:45 Welcome and Introduction to the Conference
Klaus Rose, Children's Medicines Working Party, EFGCP & Klausrose Consulting, Switzerland & Thorsten Olski, European Medicines Agency

Plenary Session 1

Chairpersons: *Klaus Rose, Children's Medicines Working Party, EFGCP & Klausrose Consulting, Switzerland & Thorsten Olski, European Medicines Agency*

09:00 Overview of 5 years (general summary of PDCO work, formulations)
Dirk Mentzer, Paediatric Committee (PDCO), European Medicines Agency (EMA)

09:30 PDD – Reflections from the drug developer's point of view
Sam Maldonado, Johnson & Johnson, USA

10:00 Questions & Answers

10:30 Coffee Break

Break-out Session

11:00 Three Parallel Breakout Working Groups, Focus on Specific Issues

Group 1: Operational issues in pediatric clinical development CRO work vs. academic clinical research networks
Chair & Introduction: *Philippa Smit-Marshall, PharmaNet, The Netherlands; Martine Dehlinger-Kremer, Paediatric Working Group European CRO Federation (EUCROF) & Omnicare Clinical Research, Germany; Marek Migdal, Children's Memorial Health Institute & European Network of Paediatric Research at the European Medicines Agency (Enpr-EMA), Poland*
Rapporteur: *Alexander Cvetcovich-Muntanola, INC Research, Spain*

Group 2: Rare conditions and the way forward: case examples of EMA-approach; practicalities
Chair & Introduction: *Birgitta Olsson, Swedish Orphan Biovitrum (SOBI), Sweden; Khazal Paradis, Genzyme, The Netherlands, Kerstin Westermark, Medical Products Agency, Sweden*
Rapporteur: *Jordi Llinares, European Medicines Agency (EMA)*

Group 3: Condition vs. indication & the EU Court of Justice decision
Chair & Introduction: *Giorgia Gavriilidou, European Medicines Agency (EMA); Genevieve Michaux, Covington & Burling, Belgium*
Rapporteur: *Gesine Bejeuhr, Pediatric Special Interest Area Community (Ped SIAC), DIA & VfA Research-Based Pharmaceutical Companies, Germany*

13:00 Lunch

Plenary Session 2

14:00 Feedback from Working Groups

Chairpersons: *Irja Lutsar, PDCO Member for Estonia & Thomas Severin, Novartis, Switzerland*

Rapporteur Group 1: *Alexander Cvetcovich-Muntanola, INC Research, Spain*
Rapporteur Group 2: *Jordi Llinares, European Medicines Agency (EMA)*
Rapporteur Group 3: *Gesine Bejeuhr, Pediatric Special Interest Area Community (Ped SIAC), DIA & VfA Research-Based Pharmaceutical Companies, Germany*

15:00 Procedural EMA changes EMA: level of details in PIP opinion, interaction PDCO – CHMP – SAWP – CAT – COMP
Paolo Tomasi, European Medicines Agency (EMA)

15:30 Coffee Break

15:50 Report & tentative answers on questions collected pre-conference: send question to paediatrics@efgcp.eu
Agnes Saint-Raymond, European Medicines Agency (EMA)

16:20 Panel discussion and general discussion

17:10 Conclusions
Klaus Rose, Children's Medicines Working Party, EFGCP & Klausrose Consulting, Switzerland

17:15 End of day 1

18:30 Social Event
Keynote Speech by *Guido Rasi, Executive Director, European Medicines Agency (EMA)* & Dinner

Thursday, 27 September 2012

Plenary Session 3

Chairpersons: *Gesine Bejeuhr, Pediatric Special Interest Area Community (Ped SIAC), DIA & Vfa Research-Based Pharmaceutical Companies, Germany & Cecile Ollivier, European Medicines Agency (EMA)*

09:00 Welcome

09:10 Extrapolation: European approach
Christoph Male, Paediatric Committee (PDCO), European Medicines Agency (EMA)

09:50 Extrapolation: FDA approach
Dianne Murphy, Food and Drug Administration (FDA), USA

10:30 Extrapolation: Industry Approach
Bruno Reigner, F. Hoffmann-La Roche, Switzerland

11:10 Coffee Break

11:40 Wahn: Model PIP
Ulrich Wahn, Pediatric Pneumology& Immunology Dpt, Charité University of Medicine Berlin, Germany

12:20 EMA: Model PIP
Richard Vesely, European Medicines Agency (EMA)

12:40 Lunch

Plenary Session 4

Chairpersons: *Irja Lutsar, PDCO Member for Estonia & Thorsten Olski, European Medicines Agency (EMA)*

13:40 Patient Organisation: PDCO member
Tsveta Schyns-Liharska, European Network for Research on Alternating Hemiplegia (ENRAH), Belgium

14:10 Patient Organisation: *Presentation to be confirmed*

14:40 Debate

15:40 Wrap up

15:50 End of the Conference