European Society for Developmental Perinatal & Paediatric Pharmacology
ESDPPP

eudipharm

UCL Université catholique de Louvain

Academic Year 2005-2006

Fourth European Course

Evaluation of medicinal products in children

Preliminary PROGRAMME

Co-ordination : Jean-Marc Husson, Gérard Pons, Jean-Paul Langhendries,
Agnès Saint-Raymond, Jean-Marie Maloteaux, Behrouz Kassaï.

DAY 1 M 0 **Thursday 9 February 2006**

8h00 - 8h30 : Registration / Welcome to participants
8h30 - 9h00 : Introduction to the course
- Catholic University of Louvain
- European diploma in pharmaceutical medicine
- Endic paediatricians and pharmacologists

DAY 1 M 1 (7h30) **Thursday 9 February 2006**

SPECIFIC ASPECTS OF PAEDIATRIC PHARMACOLOGY

1) 9h00 - 10h30 : **Jean-Paul Langhendries, Saint Vincent Clinic, Liège and UCL, Belgium** (1h30).
- Differences between adults and children : growth, development and maturation of the child.
- Impact of demographic data, prevalence of diseases and public health in children.

2) 10h30 - 11h30 : **Christopher Milne, Tufts University, Boston, USA** (1h00)
- Impact of pharmacoeconomics on children therapy.

11h30 - 12h00 BREAK

3) 12h00 - 13h00 : **Anders Rane, Huddinge University, Huddinge, Sweden** (1h00)
- Pharmacokinetics and pharmacodynamics (PK/PD) changes in children during maturation and diseases.

13h00 - 14h00 LUNCH

4) 14h00 - 15h00 : **Evelyne Jacqz-Aigrain, Robert Debré Hospital, Paris, France (**1h00)
- Prospects of pharmacogenomics in paediatric pharmacology.

5) 15h00 - 16h00 : **Hannsjörg Seyberth, University of Marburg, Marburg, Germany** (1h00)
- Main diseases unique to children requiring a specific drug evaluation.

16h00 - 16h30 BREAK

6) 16h30 - 17h30 : **Maurizio Bonati or Antonio Clavenna, Instituto Mario Negri, Milan, Italy** (1h00**)**
- Impact of compliance and medication errors/misuse in children, the role of proper information to children and parents.

7) 17h30 - 18h30 : **Gérard Pons, Saint Vincent de Paul Hospital, Paris, France** (1h00)
- Extrapolability to children of side effects in adults.
- Potential long term side effects of drugs related to exposure during growth and maturation.

DAY 2 M 2 (6h00) Friday 10 February 2006

SPECIFIC ISSUES RELATED TO DRUG DEVELOPMENT AND DRUG USE IN CHILDREN

1) 8h30 - 9h30 : **Claudia Gray, University of Nottingham, Derby, UK** (1h00)

- Extent of unlicenced and off-label use of medicinal products in children.

2) 9h30 - 10h30 : **Tony Nunn, Royal Liverpool Children's NHS Trust, Liverpool, UK** (1h00)

- The needs for pharmaceutical forms of medicinal products adapted to children.

10h30 - 11h00 BREAK

3) 11h00 - 12h00 : **Stephen Evans, London School of Hygiene & Tropical Medicine, UK** (1h00)

- Manage the specific aspects of pharmacovigilance in children.

12h00 - 13h00 LUNCH

4) 13h00 - 14h00 : **Daniel Vasmant, Sanofi-Aventis Pharma S.A., Paris, France** (1h00)

- Rationale, barriers and opportunities for developing a pediatric medicine : a research based pharmaceutical industry approach.

5) 14h00 – 15h00 : **Ronald Kurz, University of Graz, Austria** (1h00)

- Specific ethical issues concerning clinical trials in children including the use of placebo and obtention of consent form.
- Main cultural differences within Europe.

15h00 - 15h30 BREAK

7) 15h30 - 16h30 : **José Grosswasser, University Hospital Reine Fabiola, Brussels, Belgium** (1h00)

- Problems related to drug investigation in children faced by an Ethical Committee

DAY 3 M 3 (6h00) Saturday 11 February 2006

SPECIFIC ASPECTS OF CLINICAL TRIALS AND POSTMARKETING SURVEILLANCE IN CHILDREN

1) 8h30 - 10h00 : **Agnès Saint-Raymond, EMEA, London, UK** (1h30)

- Directive on GCP and clinical trials (basic and appended texts) : specific issues concerning clinical research in children.
- ICH E 11 note for guidance.
- Forthcoming EU and existing US legislative incentives.

2) 10h00 - 11h00 : **Josep Torrent-Farnell, EMEA/COMP, UK and Autonomous University of Barcelona, Spain** (1h00)

- Rare diseases, orphan drug and how to develop an orphan drug : Academic and EMEA/COMP viewpoints.

11h00 - 11h30 BREAK

3) 11h30 - 12h30 : **Susan Trainor, Brussels, Belgium** (1h00)

- Quality assurance system and site management organization/SMO for the investigational team and other partners

12h30 - 13h30 LUNCH

4) 13h30 - 15h00 : **Beatriz Silva - Lima, University of Lisbon, Portugal and EMEA/CPMP/Safety WP, UK** (45mn)

- Required preclinical studies for the marketing authorization of a new medicinal product to be used in children : the authorities viewpoint.

Gerd Bode, Altana Pharma, Hamburg, Germany (45mn)

- Preclinical studies for a new drug application in children : industry strategy including the CTD.

15h00 - 15h30 BREAK

6) 15h30 – 16h30 : **Gérard Pons, Saint Vincent de Paul Hospital, Paris, France** (1h00)

- Explain and implement the methodological and technical specifications of clinical trials in children, including placebo effect, choice and assessment of good endpoints.

DAY 4 M 4 (7h30) Wednesday 29 March 2006

PRE/PERINATAL DRUG EVALUATION AND USE

1) 8h15 - 9h15 : **Klaus Olejniczak, BfArM, Bonn, Germany** (1h00)

- Drug evaluation by Regulatory Authorities and specific issues linked to their use during pregnancy : viewpoint of the toxicologist.

2) 9h15 - 10h15 : **Sandra Kweder, FDA, Rockville, USA** (1h00)

- Drug evaluation by Regulatory Authorities and specific issues linked to their use during pregnancy : product information/labelling.
- Questioning clinical trials on medicinal products possible during pregnancy ?

10h15 - 10h45 BREAK

3) 10h45 - 11h45 : **Donald Mattison, National Institute of Child Health and Human Development, Rockville, MD, USA** (1h00)

- Principles and methodological issues on the evaluation of placental drug transfer.

4) 11h45 - 12h45 :**Elisabeth Elefant, Trousseau Hospital, Paris, France** (1h00)

- Explain the treatment of pregnant women.
- Risk of drug exposure at different stages of pregnancy : consequences for drug use in pregnant women.

5) 12h45 - 13h15 :Moderator **Jean-Marc Husson**, Eudipharm, Lyon, France (30 mn)
Panel discussion with **Elisabeth Elefant, Donald Mattison, Sandra Kweder**, **Klaus Olejniczack, Gérard Pons,** on

- "Risk of drug exposure at different stages of pregnancy

13h15 - 14h15 LUNCH

6) 14h15 - 14h45 : **Gérard Pons, Saint Vincent de Paul Hospital, Paris, France** (30 mn)

- Principles and methodological issues on the evaluation of drug transfer into breast milk. Consequences for breast fed children.

7) 14h45 - 15h45 :**Michael D. Reed, Rainbow Babies & Children's Hospital, Cleveland, U.S.A.** (1h00)

- Different clinical situations related to foetal drug therapy. Methodological issues on drug evaluation in these situations.

15h45 - 16h15 BREAK

8) 16h15 - 17h45 : **Corinne Hubinont, St Luc Hospital, Brussels, Belgium** (1h30)

- Foetal drug therapy : two clinical situations : available evidence-based data.

DAY 5 M 5 (7h30) Thursday 30 March 2006

DRUG EVALUATION IN VARIOUS SPECIFIC THERAPEUTIC AREAS

1) 8h30 - 10h00 : **Catherine Chiron, Necker Hospital, Paris, France** (1h30 mn)

- Specific aspects of epilepsia in children, differences with adults.

2) 10h00 - 11h30 : **Eric Boccard (Bristol Myers Squibb), Paris, France** (1h30)

- Specific aspects of pain in children, differences with adults.

11h30 – 12h00 BREAK

3) 12h00 - 13h30 : **Academics TBF** (1h30)

- Specific aspects of cystic fibrosis in children, differences with adults, symptomatic treatment, genetherapy.

13h30 - 14h30 LUNCH

4) 14h30 - 16h00 : **Gilles Vassal, Institut Gustave Roussy, Villejuif, Paris, France** (1h30)

- Specific aspects of evaluation in cancer diseases in children.

16h00 – 16h30 BREAK

5) 16h30 - 18h00 : **Etienne Sokal, U.C.L., Brussels, Belgium** (1h30)

- Viral hepatitis in children : natural history, specificities of evaluation and state of the art of the evaluation of medicinal products.

DAY 6 M 6 (6h00) Friday 31 March 2006

DRUG EVALUATION IN A SPECIFIC THERAPEUTIC AREA IN CHILDREN. STATE OF THE ART. PROTOCOL DESIGN (WORKSHOP TO BE CONTINUED…)

1) 9h00 - 11h00 : **Bart van Overmeire, Antwerpen University Hospital, Belgium** (2h00)

- Specific aspects of drug evaluation in ductus arteriosus in neonates.

11h00 - 11h30 BREAK

2) 11h30 - 13h30 : **Bart van Overmeire, Antwerpen University Hospital, Belgium and TBF** (2h00)

Practical training on the design of one specific protocol with its CRF in a therapeutic area : ductus arteriosus in neonates.

- ***Position of the specific problem*** : state of the art through evidence based medicine on drug evaluation in ductus arteriosus.
- ***Identification of all ethical, methodological, regulatory issues*** relevant to the design of the study protocol/CRF.

13h30 - 14h30 LUNCH

3) 14h30 - 16h30 :**TBF** (2h00)

- Practical training on the design of one specific protocol with its CRF, on drug in ductus arteriosus.

DAY 7 M 7 (3h45) Saturday 1st April 2006

DRUG EVALUATION IN A SPECIFIC THERAPEUTIC AREA IN CHILDREN. STATE OF THE ART. PROTOCOL DESIGN (WORKSHOP CONTINUED…)

Co-ordination by **Bart van Overmeire & TBF Gérard Pons and Jean-Marc Husson** (2h00)

8h30 - 10h30 - Drafting the protocol and the CRF by different students working groups, *continued …*

10h30 - 11h00 BREAK

11h00 - 12h30 - Reports by different students working groups (1h30).

12 h30 - 12h45 : Conclusions on the meeting : Gérard Pons, Jean-Marc Husson (15mn).