



Academic Year 2009-2010

Eighth European Course

Evaluation of Medicinal Products in Children

Module on drug evaluation in children

ORGANIZING COMMITTEE

- Chairperson : - Gerard Pons (Paris V University, France)
- Coordination : - Agnes Saint-Raymond (EMA, London, UK)
 - Jean-Paul Langhendries (St Vincent Clinic, Liege, Belgium)
 - Behrouz Kassaï (Eudipharm, Lyon, France)
 - Jean-Marc Husson (Eudipharm, Paris, France)
- Organizing Committee members :
 - Karel Allegaert (Leuven University, Belgium)
 - John van den Anker (Sophia Children's Hosp., Rotterdam, NL)
 - Philippe Augere (Pierre Fabre, Castres, France)
 - Elisabeth Autret-Leca (Tours University, France)
 - Mati Berkovitch (Health Gov. Dpt, Israël)
 - Maurizio Bonati (Mario Negri Instituto, Milan, Italy)
 - Daniel Brasseur (EMA, London, UK)
 - Imti Choonara (Nottingham University, UK)
 - Soizic Courcier-Duplantier (GSK, Paris, France)
 - Tibor Ertl (Aok, Pecs, Hungary)
 - Cécile Grosskopf (Produits Roche, Neuilly/Seine, France)
 - Kalle Hoppu (Helsinki University, Finland)
 - Evelyne Jacqz-Aigrain (Paris VI University, France)
 - Betty Kalikstad (Rikshospitalet, Oslo, Norway)
 - Ronald Kurz (CESP, Graz, Austria)
 - Stephanie Laër (Düsseldorf University, Germany)
 - Catherine Lassale (LEEM, Paris, France)
 - Murray Lumpkin (FDA, USA)
 - Bart van Overmeire (University Hospital, Antwerpen, Belgium)
 - Laurent Perret (Servier, Paris, France)
 - Yannick Pletan (Pfizer, Paris, France)
 - Anders Rane (Karolinska Institute, Stockholm, Sweden)
 - Josep Torrent-Farnell (Autonomous University Barcelona, EMA, London, UK)

Year 2009 – 2010 - OBJECTIVES

M 1 SPECIFIC ASPECTS OF PAEDIATRIC PHARMACOLOGY

(5H30)

- Understand differences between adults and children : growth, development and maturation of the child (1h30)
- Understand the specificities of drug evaluation in the main diseases unique to children (1h00)
- Drug kinetics and effects. Understand the influence of maturation on PK/PD relationship in children (1h00)
- Understand the impact of pharmacogenomics during development (1h00)
- Understand why there are side effects occurring specifically in children. Explain the specific aspects of side effects of drugs in children (1h00).

M 2 SPECIFIC ISSUES RELATED TO CLINICAL TRIALS AND DRUG USE IN CHILDREN

(7H00)

- Define and be aware of the extent of unlicensed and off-label use of medicinal products in children (1h00)
- Identify the needs for drug formulations adapted to children (1h00)
- Manage the specific aspects of pharmacovigilance in children (1h00)
- How to develop Orphan drugs : Academic & EMEA viewpoints ? (1h00)
- How to inform on the Implementation of the Paediatric Regulation (1h00)
- Understand the specific ethical issues in children including the use of placebo and obtention of consent form
 - Describe the directive on GCP and clinical trials. Be aware of the EU existing legislative incentives.
 - Determine the criteria Medicine Agencies are applying to authorize new drugs in children (1h30)

M 3 METHODOLOGICAL ASPECTS RELATED TO CLINICAL TRIALS AND DRUGS USE IN CHILDREN

(7h30)

- Explain and implement the methodological and technical specifications of clinical trials in children including placebo effect, choice and assessment of good endpoints (1h00).
- Understand the potential role of pharmacokinetic-pharmacodynamic modelling in drug development in children (1h00)
- Implement the modelling of the maturation of drug metabolic pathways to drug development in children (1h30)
- Explain the interest of modelling in dose-finding.
- Understand the place of sequential methodological approaches in drug development in children : phase 1 to 3 studies (1h00)
- Explain the contribution of meta-analysis in drug development in children (1h00)
- Understand the specificities of pharmacoepidemiology in children (1h00)

M 4 SPECIFIC ISSUES RELATED TO DRUG DEVELOPMENT AND POSTMARKETING SURVEILLANCE IN CHILDREN (3h30)

- Understand the rationale and place of preclinical studies in children during drug development (1h00)
- Identify the process used to develop a paediatric drug in the Pharmaceutical Industry (1h00)
- Understand the process of elaboration of the Paediatric Investigation Plan and understand the reasons why to initiate drug development in children at different phases of drug development in adults : the regulatory (EMA/CPMP) view point (scientific advice...) and industry strategy (1h30).

M 5 PRE/PERINATAL DRUG EVALUATION AND USE (6h30)

- Review the different clinical situations related to foetal drug therapy. Elaborate on the methodological issues on drug evaluation in these situations (1h30)
- Understand the principles and identify the methodological issues on the evaluation of placental drug transfer (1h00)
- Writing product information/labeling, questioning clinical trials on medicinal products possible during pregnancy (1h30)
- Explain the treatment of pregnant women : Assess the risk of drug exposure at different stages of pregnancy. Evaluate the consequences for drug use in pregnant women (1h00)
- Discussion about 3 cases of drug use in pregnant women (1h30)

M 6 DRUG EVALUATION IN VARIOUS SPECIFIC THERAPEUTIC AREA IN CHILDREN. STATE OF THE ART. DESIGN OF A PAEDIATRIC INVESTIGATION PLAN (WORKSHOP) (7h00)

- Review the state of the art on the evaluation of medicinal products of various classes in children through evidence-based medicine (1h00)
- Proceed to the identification of all ethical, methodological and regulatory issues linked to the design of 2 specific Paediatric Investigation Plans (PIP) (1h00)
- How to design 2 specific Paediatric Investigation Plans (PIP) in different therapeutic areas (5h00)
 - ✓ Instructions (1h30)
 - ✓ Drafting 2 Paediatric Investigation Plans (PIP) P.I.P. (2h00)
 - ✓ Students' reports (1h30)

M 7 DRUG EVALUATION IN A SPECIFIC THERAPEUTIC AREA IN CHILDREN. STATE OF THE ART (9h00)

- Describe experience of a clinical study within the French Paediatric Network (1h30)
 - Describe specific aspects of drugs for pain in children (1h30)
 - Describe specific aspects of clinical practice of drugs of the ductus arteriosus in neonates (1h30)
 - Describe specific aspects of evaluation of vaccines in children (1h30)
 - Describe specific aspects of evaluation of anticancer drugs in children (1h30)
 - Describe specific aspects of drug evaluation in epilepsy in children (1h30)
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Year 2009 - 2010

MODULES ON EVALUATION OF MEDICINAL PRODUCTS IN CHILDREN

TIME-SCHEDULE (2 periods)

- **M 1 Tuesday** **February 23rd 2010**
- **M 2 Wednesday** **February 24th 2010**
- **M 3 Thursday** **February 25th 2010**
- **M 4 Friday** **February 26th 2010**

- **M 5 Tuesday** **March 23th 2010**
- **M 6 Wednesday** **March 24th 2010**
- **M 7 Thursday** **March 25th 2010**
- **M 7 Friday** **March 26th 2010**

VENUE

**UNIVERSITY RENÉ DESCARTES PARIS V – COCHIN HOSPITAL – ROOM DIEULAFOY
(27, rue du Faubourg Saint Jacques – 75014 Paris)**