

Academic Year 2009-2010

Eighth European Course Evaluation of Medicinal Products in Children

VENUE : 27, rue du Faubourg St Jacques, 75014 Paris, France

PRELIMINARY PROGRAMME (46h15)

Co-ordination : Jean-Marc HUSSON, Behrouz KASSAI, Jean-Paul LANGHENDRIES, Gerard PONS, Agnes SAINT-RAYMOND

DAY 1 M 0

Tuesday 23 February 2010

13h00 - 13h30 : Registration / Welcome to participants

13h30 - 13h45 : Introduction to the course

- Paris V University (Rene Descartes Medical School)
- European Diploma in Pharmaceutical Medicine
- ENDIC paediatricians and pharmacologists (European Society for Developmental, Perinatal and Paediatric Pharmacology)

DAY 1 M 1 (4h30)

Tuesday 23 February 2010

SPECIFIC ASPECTS OF PAEDIATRIC PHARMACOLOGY

1) 13h45 - 15h15 : **Jean-Paul Langhendries, St Vincent Hospital, Liege 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) 15h15 - 16h15 : **Kalle Hoppu, Helsinki University, Helsinki, Finland (1h00)**

- Main diseases unique to children requiring a specific drug evaluation.

16h15 - 16h45 BREAK

3) 16h45 - 17h45 : **Anders Rane, Karolinska Institute at Karolinska University, Sweden (1h00)**

- Pharmacokinetics and pharmacodynamics (PK/PD) changes in children during maturation and diseases

4) 17h45 - 18h45 : **Evelyne Jacqz-Aigrain, Robert Debre Hospital, Paris, France (1h00)**

- Prospects of pharmacogenomics in paediatric pharmacology.

DAY 2 M 1 (1h00)**Wednesday 24 February 2010****SPECIFIC ASPECTS OF PAEDIATRIC PHARMACOLOGY**

1) 8h30 - 9h30 : **Gerard Pons, St Vincent de Paul Hospital, Paris V University, 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 (7h00)**Wednesday 24 February 2010****SPECIFIC ISSUES RELATED TO CLINICAL TRIALS AND DRUG USE IN CHILDREN**

2) 9h30 - 10h30 : **Imti Choonara, Nottingham University, Derby, UK (1h00)**

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

10h30 - 11h00 BREAK3) 11h00 - 12h00 : **Tony Nunn, Royal Liverpool Children's NHS Trust, Liverpool, UK (1h00)**

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

4) 12h00 - 13h00 : **Dirk Mentzer, Paul Ehrlich Institute, Langen, Germany (1h00)**

- Pharmacovigilance and Risk Management Plan in children

13h00 - 14h00 LUNCH5) 14h00 - 15h00 : **Agnes Saint-Raymond, EMEA, London, UK (1h00)**

- Rare diseases, orphan drug and how to develop an orphan drug : academic and EMEA/COMP viewpoints.
- Methodology of CTs in MDD for orphan drugs : experience of COMP

6) 15h00 - 16h00 : **Daniel Brasseur, EMEA/CPMP, London, U.K. (1h00)**

- The Implementation of the Paediatric Regulation. Tasks and Mandates of the different parties

16h00 - 16h30 BREAK7) 16h30 - 18h00 : **Agnes Saint-Raymond, EMEA, London, UK (1h30)**

- Ethical, regulatory and legal framework of the drug development in children.
- Specific ethical issues concerning clinical trials in children including the use of placebo and obtention of consent form.
- Integrating Ethics in EU Research.

1) 8h30 - 9h30 : **Gerard Pons, St Vincent de Paul Hospital, Paris V University, France** (1h00)

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

2) 9h30 - 10h30 : **Jean-Louis Steimer, Steve Kern, Novartis Pharma, Basel, Switzerland** (1h00)

- Place of pharmacokinetics and pharmacodynamics modelling in drug development in children. Examples

10h30 - 11h00 BREAK

3) 11h30 - 13h00 : **Amin Rostami-Hodjegan, Royal Hallamshire Hospital, Sheffield, U.K.** (1h30)

- Modelling of the influence of growth and maturation in drug development in children. Example of the maturation of drug metabolism.

13h00 - 14h00 LUNCH

4) 14h00 - 15h00 : **Frank Bretz, Novartis Pharma, Basel, Switzerland** (1h00)

- Modelling in dose-finding studies in children.

5) 15h00 - 16h00 : **Sarah Zohar, Saint Louis Hospital, Paris, France** (1h00)

- Place of sequential methodological approaches in phase 1-2 and 3 clinical studies during drug development in children.

16h00 - 16h30 BREAK

6) 16h30 - 17h30 : **Behrouz Kassaï, Laënnec University, Lyon, France** (1h00)

- Clinical cases : meta-analysis in paediatrics.

7) 17h30 - 18h30 : : **Lamiae Grimaldi-Bensouda, La-Ser, Paris, France**
Martin Chalumeau, St Vincent de Paul Hospital, Paris, France (1h00)

- Specific methodological aspects in pharmaco-epidemiology in children.

DAY 4 M 4 (3h30)

Friday 26 February 2010

SPECIFIC ASPECTS RELATED TO DRUG DEVELOPMENT AND POSTMARKETING SURVEILLANCE IN CHILDREN

1) 9h00 - 10h00 : **Beatriz Silva-Lima, Lisbon University, Portugal & EMEA, CPMP/Safety WP, UK (1h00)**

- Required preclinical studies for the marketing authorization of a new medicinal product to be used in children (including juvenile animal models) : the authorities viewpoint.
- Preclinical studies for a new drug application in children : industry strategy

2) 10h00 - 11h00 : **Khazal Paradis, Genzyme Europe, Naarden, The Netherlands (1h00)**

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

11h00 - 11h30 BREAK

3) 11h30 – 13h00 : **Paolo Tomasi, EMEA, London, U.K.(1h30)**

- Paediatric Investigation Plan
- Reasons to initiate drug development in children at different phases of drug development in adults : the regulatory (EMEA/CPMP) viewpoint (scientific advice...)

1) 12h30 - 14h00 : **Michael D. Reed, Akron Children's Hospital, Akron, U.S.A.** (1h30)

- Different clinical situations related to fetal drug therapy. Methodological issues on drug evaluation in these situations and available evidence-based data. (1h00)

Case study (30 min)

2) 14h00 - 15h00 : **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.

15h00 - 15h30 BREAK

3) 15h30 - 17h00 : **Donald Mattison, National Institute of Child Health and Human Development, Rockville MD, USA** at **Sandra Kweder's** request (**FDA, Washington, U.S.A.**) (1h30)

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

4) 17h00 - 18h00 : **Elisabeth Elefant, Trousseau Hospital, Paris, France,** at (1h00)

- Drug therapy in pregnant women.
- Risk of drug exposure at different stages of pregnancy : consequences for drug use in pregnant women.

5) 18h00 - 19h30 : Panel discussion with : **Elisabeth Elefant, Donald Mattison, Gerard Pons, Michael D. Reed** (1h30)

Case studies : 3 cases / 6 groups :

- **Case 1** : Drug exposure during early pregnancy
- **Case 2** : Drug exposure during late pregnancy
- **Case 3** : Drug exposure during before delivery

DAY 6 M 6 (7h00)

Wednesday 24 March 2010

**DRUG EVALUATION IN VARIOUS SPECIFIC THERAPEUTIC AREAS IN CHILDREN.
STATE OF THE ART. DESIGN OF A PAEDIATRIC INVESTIGATION PLAN (WORKSHOP)**

1) 8h30 - 10h30 : **Sophie Fornairon, A.F.S.S.A.P.S., Saint Denis, France** (2h00)

Practical training on the design of a Paediatric Investigation Plan (PIP) in two different therapeutic areas (2 subsequent case studies) according to the following sequence : (lecture)

- **Position of the specific problem** : state of the art through evidence based medicine on drug evaluation in these two specific therapeutic areas.
- **Identification of all ethical, methodological, strategic, regulatory issues** relevant to the design of these two specific Paediatric Investigation Plans (PIP).

10h30 - 11h00 BREAK

2) 11h00 - 12h30 : **Sophie Fornairon, A.F.S.S.A.P.S., Saint Denis, France** (1h30)

- Practical training on the design of two specific Paediatric Investigation Plans (PIP) (instructions).

12h30 - 13h30 LUNCH

3) 13h30 - 15h30 : Co-ordination by **Sophie Fornairon, Gerard Pons** (2h00)

- Drafting the Paediatric Investigation Plan n°1 (P IP) by students in separate groups
- Drafting the Paediatric Investigation Plan n°2 (P IP) by students in separate groups

15h30 - 16h00 BREAK

4) 16h00 - 17h30 : Co-ordination by **Sophie Fornairon, Gerard Pons** (1h30)

- Reports by the different student working groups

DAY 7 M 7 (6h00)

Thursday 25 March 2010

DRUG EVALUATION IN A SPECIFIC THERAPEUTIC AREA IN CHILDREN. STATE OF THE ART.

1) 10h30 - 12h00 : **Behrouz Kassaï, Laënnec University, Lyon, France** (1h30)

- Lessons from the experience of the French Paediatric Network (RIPPS), on the basis of one specific clinical study.

2) 12h00 - 13h30 : **Chantal Wood, Robert Debre Hospital, Paris, France** (1h30)

- Specific aspects of the evaluation of pain in children, differences with adults

13h30 – 14h30 LUNCH

3) 14h30 - 16h00 : **Bart van Overmeire, Antwerpen University Hospital, Belgium** (1h30)

- Specific aspects of drug evaluation in ductus arteriosus in neonates

4) 16h00 - 17h30 : **Marie-Claude Bonnet, Sanofi-Pasteur, Lyon, France** (1h30)

- Specific aspects of the evaluation of vaccines in children

DAY 8 M 7 (3h15)

Friday 26 March 2010

DRUG EVALUATION IN A SPECIFIC THERAPEUTIC AREA IN CHILDREN. STATE OF THE ART.

1) 9h00 - 10h30 : **Gilles Vassal, Gustave Roussy Institute, Villejuif, France (1h30)**

- Specific aspects of the evaluation of drugs in cancer diseases in children.

2) 10h30 - 12h00 : **Catherine Chiron, Necker Hospital, Paris, France (1h30)**

- Specific aspects of the evaluation of epilepsy in children, differences with adults.

3) 12h00 - 12h15 : Conclusions of the meeting : **Gerard Pons (15mn).**

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Evaluation of Medicinal Products in Children

23, 24, 25, 26 February 2010 - 23, 24, 25, 26 March 2010

GENERAL INFORMATION

VENUE – 27, RUE DU FAUBOURG ST JACQUES – 75014 PARIS

The lectures will take place :

Lecture room DIEULAFOY in Cochin Hospital : **27, rue du Faubourg St Jacques, 75014 Paris,**
France. RER line B station : Port Royal or Bus # 38 Port Royal stop.

BADGES

On registration you will receive a badge. You will have to keep it from the beginning to the end of the lectures. It gives you access to the lecture rooms.

LANGUAGE

The lectures language is English

COFFEE BREAKS

Coffee and tea are served outside the room.

ACCESS

FROM CHARLES DE GAULLE AIRPORT :

ROISSYBUS : depending on traffic from 40 min to 2 h. From the airport to the Center of Paris (Opera),
Cost : ±. 9,10 €

By taxi : depending on traffic from 40 min to 2 h. The cost will be, accordingly, from ±.45 to 100 €

By Train (RER B) : Direction "St Remy les Chevreuse" to the center of Paris (e.g. Saint Michel or Châtelet station up to Port Royal Station) connection with the metro. Cost : ±. 8,50 €.

FROM ORLY AIRPORT :

ORLYBUS : depending on traffic : from 20 min to 1 h ("Denfert Rochereau" station). Cost : ± 6,40 €.

By taxi : depending on traffic : from 20 min to 1 h ("Denfert Rochereau" station). The cost will be, accordingly, from 25-30 to 60 €

By Train : ORLYVAL 8 min from the airport to Antony station where connection with RER B, Direction "Charles de Gaulle", first stop in Paris to be connected with the metro : "Denfert Rochereau" station, 15 min. Cost : ±. 9,85 €.

FROM TRAIN STATIONS :

- **North Station so called "Gare du Nord" :**

Eurostar and Thalys trains arrival.

Take RER B Direction "St Remy les Chevreuse" up to Port Royal Station

- **East Station so called "Gare de l'Est" :**

Go to Gare du Nord (2 min walking distance) then cf above

FOR ANY OTHER INFORMATION, PLEASE CONTACT : Nadine PONS

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