GIANNI BENZI
PHARMACOLOGICAL RESEARCH
FOUNDATION

PROGRAMME

EUROPEAN CENTRALISED PROCEDURE AND PAEDIATRIC REGULATION

Pavia, 2-4 September, 2008

Fondazione Salvatore Maugeri Via Maugeri, 4 – Pavia (Italy)

1st Topic: European Centralised procedure from A to Z

2 September 2008

Chairmen : Eugenio Muller - Enrico Bosone

9.30 **Introductory Remarks**
Angiolino Stella, Chancellor of the University of Pavia
Marcello Imbriani, Scientific Director S. Maugeri Foundation
Guido Rasi, Director of AIFA
Sergio Dompé, President of Farmindustria

10.00 **Introduction**
The Gianni Benzi Foundation role and perspective
Vittorio Silano

Chairmen : Mario Cazzola - Cesare Fieschi

10.30 **Opening Remarks**
The EMEA and the future of pharmaceuticals for human use
Thomas Lönngren
11.00 A European Network for Education in the Regulatory Affairs context
Pino Nisticò

11.30 *Coffee Break*

Chairmen : Vittorio Silano - Walter Bianchi

11.45 **Tools for development of innovative drugs: Scientific Advice (SA) and Protocol Assistance (PA)**
- When a SA or a PA are convenient
- How they work
- Many advantages and few warnings

Hans-Georg Eichler /Jacques Mascaro /Jean-Pierre Osselaere

12.45 **Presubmission activities**
- When they are appropriate
- What you need to know

Jean-Pierre Osselaere/Henk Schuring/ Hans-Georg Eichler

14.00 *Lunch*

Chairmen : Walter Bianchi - Marcello Tonini

15.00 **The application and the first period of the Assessment**
- Strategies in preparing the e-CTD
- How to work with EMEA

Henk Schuring/Pino Nisticò

16.00 *Coffee break*

16.15 **Objections and answers**
- Minor and major objections
- Main reasons for major objections
- How to answer to the objections

Jacques Mascaro/Henk Schuring/Pino Nisticò

17.15 **Q&A**
All speakers

3 September 2008

Chairmen : Enrico Bosone - Jean-Pierre Osselaere

9.00 **Oral explanation and dialogue with Rapporteur, Co-Rapporteur and Project Manager**
- How to understand the objections
- Examples of positive dialogue
- 25% of withdrawals: when and why
- Orphan drugs and paediatric indications: how the different Committees work together

Chris Walker/Eric Abadie

10.00 **After the Opinion: commitments and appeals**
- Main commitment for oncologic drugs
- Examples of successful appeals

Chris Walker/Eric Abadie

11.00 *Coffee Break*

Chairmen : Jean-Pierre Osselaere - Enrico Bosone

11.15 **From the Opinion to the Decision**
- Steps between the Opinion and the Decision
- Labelling for Europe

Chris Walker

11.45 **Remarks on Centralised Procedure**
- CHMP and Centralised procedure: challenges for the future

Eric Abadie

12.45 **Q&A and final remarks**
All speakers

13.15 *Lunch*

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2nd Topic: The Paediatric Regulation Challenges

3 September 2008

Chairmen : Paola Baiardi – Mariana Catapano

14.30 **Introductory remarks on the Paediatric Regulation**
- The role of the Paediatric Committee (PDCO): PIP submission, waivers and deferrals
Daniel Brasseur
- The Network of Paediatric Networks envisaged in the Paediatric Regulation in supporting paediatric drugs development
Paolo Rossi
- The Task-force in Europe for Drug Development for the Young (TEDDY) role and mission
Adriana Ceci

15.30 **Early clinical research**
- Strategies to identify and mitigate risks for first-in-human clinical trials with investigational medicinal products
Oscar Della Pasqua
- Role of pharmacokinetics in the development of medicinal products in the paediatric population
Oscar Della Pasqua
- Introduction to population PK/PD, developmental PK/PD and modelling in paediatric clinical pharmacology
Oscar Della Pasqua

16.30 *Coffee Break*

Chairmen : Jean-Pierre Osselaere – Domenico Criscuolo

16.45 **Pharmacogenetics and Pharmacogenomics in paediatric drug developmental phase**
- Impact of PGx/PGt in children: relevant aspects
Achille Iolascon/Roberta Russo
- PGx/PGt in children in Europe: where and how
Oscar Della Pasqua

17.15 **Preclinical issues**
- Paediatric specificity for non-clinical tests
- Guideline on the need for non-clinical testing in juvenile animals on human pharmaceuticals for paediatric indications
Annarita Meneguz/Marcello Tonini

17.45 **Q&A**
All speakers

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4 September 2008

Chairmen : Ian Wong - Paolo Paolucci

9.00 **Quality and paediatric formulations**
- Development of paediatric formulations
Jean Pierre Osselaere/Henk Schuring
- Old Drugs/New formulations
Anu Tummavuori

9.40 **Methodology in conducting clinical trials in children**
- Formulations of experimental drugs
Jean Pierre Osselaere/Henk Schuring
- How to apply for a paediatric clinical trial
Domenico Criscuolo
- Regulatory aspects and PIP application
Mariana Catapano
- Case Studies: focus on specific therapeutic areas- Vaccines
Eddie Reilly

11.00 *Coffee Break*

Chairmen : Maria Mellado Pena - Anu Tummavuori

11.15 **Paediatric Research in Europe**
- Current status of paediatric research
Paola Baiardi
- Ethical concerns in paediatric clinical trials
Annagrazia Altavilla
- Paediatric Research Costs and Benefits
Massimo Iacobelli

12.15 **Q&A**
All speakers

13.00 Lunch

Chairmen : Emilio Perucca - Eddie Reilly

14.00 **Pharmacoepidemiology and Pharmacovigilance**
- How to collect paediatric data in Europe
Miriam Sturkenboom
- Could pharmacepidemiology guide the drug developmental plan?
Anu Tummavuori
--Could pharmacoepidemiology guide the SMEs and academic paediatric research?
Ian Wong
- Pharmacovigilance and management risks activities addressing the paediatric specificity
Katia Verhamme

Chairmen : Adriana Ceci - Enrico Bosone

15.30 **Case Studies: Focus on specific therapeutic areas**
- Neonatology *Marek Migdal*
- Oncology *Paolo Paolucci*
- Respiratory diseases *Eugenio Baraldi*
- Infectious diseases *Maria Mellado Peña*
- Epilepsy *Emilio Perucca*

17.00 **Q&A, Tests and Conclusion**

SPEAKERS' LIST

Eric Abadie, Président du CHMP, EMEA and Conseiller scientifique auprès du Directeur Général, Direction Générale, Afssaps

Annagrazia Altavilla, Assistance Publique des Hôpitaux de Marseille (APHM)-Espace Ethique Méditerranéen

Paola Baiardi, Director, Consortium for Biological and Pharmacological Evaluations (CVBF)

Eugenio Baraldi, Department of Paediatrics Chief of the Unit of Allergy and Respiratory Deseases University of Padova

Walter Bianchi, President, Italian Society of Regulatory Affairs (SIAR)

Enrico Bosone, Director Regulatory Affairs & Compliance, Celgene Srl and SIAR Member of MB

Daniel Brasseur, Chair, Paediatric Committee (PDCO), EMEA

Mariana Catapano, Head of Regulatory Affairs, Consortium for Biological and Pharmacological Evaluations (CVBF)

Mario Cazzola, Pro-Chancellor University of Pavia, President Consortium for Biological and Pharmacological Evaluations (CVBF)

Adriana Ceci, President, 'Gianni Benzi' Pharmacological Research Foundation and TEDDY NoE Coordinator

Domenico Criscuolo, CHIEF Medical Officer, Creabilis Therapeutics Spa, and IFAPP President

Oscar Della Pasqua, Assistant Professor, Center for Drug Research, Leiden University and Clinical Pharmacology & Discovery Medicine, GlaxoSmithKline

Sergio Dompé, President of Farmindustria

Hans-Georg Eichler, Professor, Vienna University School of Medicine, Senior Medical Officer, EMEA

Cesare Fieschi, Professor of Neurology , University La Sapienza di Roma

Massimo Iacobelli, Chief Scientific Officer, Gentium Spa

Marcello Imbriani, Fondazione S. Maugeri, Scientific Director, Pavia

Achille Iolascon, Professor, University Federico II of Naples and CEINGE- Advanced Biotechnologies

Anne Marie Li-Kwai-Cheung, Regulatory Affairs Europe, Genzyme

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Thomas Lönngren, Executive Director, European Medicines Agency -EMEA
Jacques Mascaro, Vice President Regulatory Affairs and Safety, Elan
Maria José Mellado Pena, Hospital Carlos III and TEDDY NoE-Spain Coordinator
Annarita Meneguz, Director of Biochemical Pharmacology Department, ISS
Marek Migdal, Professor, Neonatologist Children's Memorial Health Institute and member Paediatric Committee (PDCO), EMEA
Eugenio Muller, Professor of Pharmacology, University of Milan
Giuseppe Nisticò, EMEA Board and CHMP member, Director Master in "Scientific and Regulatory assessment of new drugs" Tor Vergata University – Rome
Jean Pierre Osselaere, Managing Director, EPMC Pharma
Paolo Paolucci, Professor of Paediatrics and Head Department, University of Modena and Reggio Emilia
Emilio Perucca, Professor of Clinical Pharmacology, University of Pavia
Guido Rasi, General Director of Italian Medicines Agency (AIFA)
Eddie Reilly, Head, Regulatory Affairs, Europe & International, Novartis Vaccines and Diagnostics s.r.l
Paolo Rossi, Member Paediatric Committee (PDCO),EMEA and Ospedale Bambino Gesù
Roberta Russo, University Federico II of Naples and CEINGE- Advanced Biotechnologies
Vittorio Silano, President Scientific Committee, 'Gianni Benzi' Pharmacological Research Foundation and President of the EFSA Scientific Commission
Angiolino Stella, Chancellor of the University of Pavia
Miriam Sturkenboom, Professor of Pharmaco-epidemiology, Erasmus University Medical Center
Marcello Tonini, Professor and Head Department, University of Pavia and Director Master in "Regulatory Sciences - GIANNI BENZI" University of Pavia
Anu Tummavuori, Associate Director European Regulatory Liaison, Celgene International Sarl Switzerland
Katia Verhamme, Senior researcher,Department of Medical Informatics Erasmus University Medical Center
Chris Walker, Senior Manager Regulatory Affairs, Amgen
Ian Wong, Professor of Paediatric Medicines Research, and Director Centre for Paediatric Pharmacy Research