



1/3 September 2010

Krakow • Poland

III Foresight

Training Course

***Benefit/Risk  
Assessment of  
Medicines to  
achieve shared  
objectives:  
from Research  
to Reality***

The Course is intended to re-read all the phases of drug development, with the benefit/risk ratio as a compass and primary target of the efforts of all involved in this difficult process

June 2010

Dear Colleagues,

the 'Gianni Benzi' Pharmacological Research Foundation, in collaboration with the National Medicines Institute and the Children's Memorial Health Institute of Warsaw; SIAR, the Italian Society of Regulatory Affairs ; MEGRA, the Middle European Society of Regulatory Affairs; TEDDY, the Task-force in Europe for Drug Development for the Young; the Master in Regulatory Sciences "Gianni Benzi" (University of Pavia) and the CVBF (Consortium for Biological and Pharmacological Evaluation), is organizing its III "Foresight Training Course" that will be held in Krakow next 1-3 September 2010.



The first "Foresight Training Course" was held in September 2008 and focused on the Centralised Procedure and the Pediatric Regulation. The second "Foresight Training Course" was held in September 2009 and focused on Orphan Drugs, Advanced Therapies and Paediatric Clinical Studies. Both were granted by the European Accreditation Council for Continuing Medical Education (EACCME) and were designated for 18 European CME credits for medical specialists (for more information and documents please see [www.benzifoundation.org](http://www.benzifoundation.org)).

The third course takes inspiration from the EMA Road Map and is intended to re-read all the phases of drug development, with the benefit/risk ratio as a compass and primary target of the efforts of all the actors involved in this difficult process. Please find a draft of the rationale and the program of the Course. This is the first time the Course is outside Italy: Krakow has been selected for its special meaning in the past history of Europe and in the new process of integration of Europe with eastern countries.

We sincerely hope that you will attend the Course and the dinners and social events, during which you will have the possibility for meeting many Experts and Regulators.

With our best wishes,  
Enrico Bosone

Marek Migdal

Adriana Ceci

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to achieve shared objectives:  
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## Background

To date the benefit/risk assessment has been mainly limited at the phase of the evaluation for the Marketing Authorization, but in the future it will increasingly become one of the most important parameters in the new drug development process.

In fact, in the modern development of a new drug, this evaluation already starts during the Research phase and must be repeated in all steps of the scientific and regulatory pathway, until the drug reaches the attention of the Physician who wants to use it for his Patient.

The Course has the intention to review all the phases of the drug development process, having the parameter of the continuous benefit/risk ratio as a compass and main target of the efforts of all the players involved in the developmental process: the Sponsor who needs to find promising and successful fields for investments; the Health Authorities who must guarantee the safety of the patients but who have also the responsibility to drive the efforts towards the most important clinical needs; the Researchers and the Physicians who are strongly engaged to reach a benefit for patients and to gain also an intellectual satisfaction; the Patients and the Patient Associations who request that Science helps in limiting the avoidable sufferings without distinction of race or wealth.



## Main topics

It is impossible clearly distinguishing between Research and Development: it is a continuous process. The first sessions of this Course are devoted to the Research and to the procedures for the submissions of Clinical Trial applications, taking into account the appropriate guidelines and the international and European rules.

Actual examples for an application of an orphan drug, for an IMPD preparation, for the application to the PDCO, as well as the procedures for the applications to the Ethic Committee, will be shown and discussed. Examples of B/R evaluation during this development phase will be provided.

During the process, in particular for innovative therapies, the dialogue between the Sponsor and the Health Authorities increases. Session III deals with the role of different EMA Committees involved in the risk/benefit assessment in different developmental phases.

The mechanisms regarding Scientific Advice, Protocol Assistance, IND, will be evaluated also through real examples and guidance for the operators, always having the benefit/risk assessment as leading feature which has to be measured continuously.

The core of the development process still remains the preparation of the MA dossier. In Sessions IV and V the preparation and submission will be revisited with a special care for the most relevant aspects for benefit/risk evaluation. Some concrete examples of the procedure for the assessment will be supplied and the possibility for a personalized assessment of the B/R ratio will be also discussed. This opportunity is now possible, in some cases, using, for example, the pharmacogenetic tools.

Two sessions are devoted to the benefit/risk evaluation in the post-marketing phase. Session VI deals with the Pharmacovigilance and "Risk management Plans". In this session the methods to implement an efficacious system for the B/R management plan, connected with a total Pharmacovigilance system, will be shown and discussed.

A final session is devoted at evaluating benefit/risk in the context of HTA reports. The HTA bodies, now present in many Nations, perform a comparative evaluation of the B/R ratio, taking into account also economic elements. The joint between these two activities [i.e. B/R assessment and HTA] is now under the attention of the different Institutions and will be discussed with relevant Experts.

### *Course Directors*

Enrico Bosone - SIAR representative in the Gianni Benzi Foundation Board  
Marek Migdal - Deputy Head of PICU, Children's Memorial Health Institute, Warsaw,  
and member of Paediatric Committee (PDCO), EMA

### *Scientific Committee*

Adriana Ceci - President of Gianni Benzi Pharmacological Research Foundation  
Sergio Dompé - President of Farindustria  
Michał Pirozynski - Postgraduate Medical School, Warsaw, and member of CHMP, EMA  
Walter Bianchi - President of SIAR, Italian Society for Regulatory Activities  
Beatrice Jaha - Member of MEGRA MB, Middle European Association for Regulatory Affairs  
Mariana Catapano - Director of GISF, Italian Group for Pharmacoeconomic Studies

### *Organising Secretariat*

Bożena Aukomska-Tyll, Instytut "Pomnik-Centrum Zdrowia Dziecka" - [dyr.naukowy@czd.pl](mailto:dyr.naukowy@czd.pl)  
Donato Bonifazi, Corporate Secretary Gianni Benzi Foundation - [secretary@benzifoundation.org](mailto:secretary@benzifoundation.org)

## 13.30 Opening Remarks

*E. Bosone - J. Gralinski*

## Welcome Session - Benefit/Risk in the perspective of the European Road Map and National Agencies proposals

*Chairs: M. Migdal – A. Ceci*

EMA Road Map perspectives and contribution

*A. Saint-Raymond, European Medicines Agency*

National Agencies vision and skills

*A. Cieslik, Polish Medicinal Agency*

*P. Siviero, Italian Medicinal Agency*

Pharmaceutical Industries Representatives

*G. Caruso, Farindustria, Italy*

*P. Zukowski, Sanofi-Aventis, Poland*

Patient's Association Representative

*F. Houyez, EURORDIS*

EMA legal responsibility and role in B/R Assessment

*T. Jablonski, European Medicinal Agency*

Discussion

Coffee break

## 16.30 Session 1

### **Research, development and B/R ratio**

*Chairs: P. Paolucci – A. Gyurasics*

Promote Research for SMEs in the EC-FP7 Framework

*F. Donnelly, European Commission DG-Research*

IMI Innovative Medicines Initiatives

*T. Higenbottam, Chiesi Farmaceutici*

Paediatric Priority List

*K. Connolly, European Medicines Agency;*

*Portiuncula Hospital, Ireland*

Discussion



### 08.30 Session 2

#### ***B/R assessment during the development***

Chairs: *A. Saint Raymond - M. Migdal*

Personalized therapy and improved B/R Assessment

*O. Della Pasqua, GlaxoSmithKline; Leiden University*

Assessment of IMPD for Phase 1: different critical issues for small CE vs Bio products

*A. Meneguz, Italian Institute of Health*

Jouvenile animal studies to improve B/R ratio

*J. Gallego, B. Matrot; INSERM*

How to plan and evaluate CT for small population

*P. Baiardi, Consortium for Biological and Pharmacological Evaluations*

Risk Management Strategy during development

*F. Vegni, Celgene International*

Discussion

Coffee break

### 11.00 Session 3

#### ***Risk/Benefit in the Commitees evaluation process***

Chairs: *K.Connolly – J. Gralinski*

PDCO Assessment of the B/R ratio

*D. Brasseur, PDCO Chair, European Medicines Agency*

COMP Assessment of the B/R ratio

*B. Dembowska - Baginska, COMP member EMA; Instytut Pomnik Centrum Zdrowia Dziecka*

CHMP Assessment

*M. Pirozynski, former CHMP member EMA*

The new EPAR including the CHMP evaluation process

*I. Hudson, Medicines Control Agency and CHMP*

Optimisation of consultation process CHMP/SAGs

*A. Saint-Raymond, European Medicines Agency*

Discussion

Lunch



### 14.15 Session 4

#### ***Driving incentives by making a good application***

*Chairs: D. Brasseur - E. Bosone*

IMPD for Phase 1 and procedures in Italy vs EU

*L. Lambiase, S. Paratore, Novartis Farma*

Procedure for PIP Approval

*R. Ancuceanu, PDGO and CHMP member EMA*

New Challenges for old drugs: the case of PUMA

*M. Catapano, Italian Group for Pharmacoeconomic Studies*

How to prepare and obtain an ODD

*C. Walker, Amgen*

OD rewards and strategic considerations

*C. Edfjaell, Celgene International*

Discussion

Coffee break

### 16.45 Session 5

#### ***Take advice to be successful***

*Chairs: A. Ceci – C. Walker*

Scientific advice and PA: preparation of the application

*M. Pirozynski, former CHMP member EMA*

Examples of SA in Europe

*O. Tyden, EUREDA*

Confirmatory CTs in Europe & USA: a case report

*M. Iacobelli, Gentium*

Success and failures in SA and PA

*A. Saint-Raymond, European Medicines Agency*

Discussion



## 08.00 Session 6

### **B/R Assessment after the MA**

Chairs: *M. Migdal – A. Saint Raymond*

Risk Management Plan in the B/R ratio definition

*D. Mentzer, Paul-Ehrlich-Institut; PDCO member EMA*

B/R Assessment and off-label use in children

*A. Ceci, President, Gianni Benzi Foundation; PDCO member EMA*

Pharmacoepidemiology and B/R Assessment

*M. Sturkenboom, Erasmus University Medical Center*

B/R: the AIFA Registers experience

*L. De Nigro, Italian Medicinal Agency*

B/R in the current clinical practice in paediatric oncology

*P. Paolucci, University of Modena and Reggio Emilia; PDCO member EMA*

Discussion

Coffee break

## 11.00 Session 7

### **B/R assessment and HTA**

Chairs: *I. Hudson - M. Pirozynski*

National Agencies Experiences

*P. Siviero, Italian Medicines Agency  
R. Ancuceanu, Romanian Medicines Agency*

HTA methodology for Medicines

*M. Marchetti, Università del Sacro Cuore*

HTA and patients rights

*F. Houyez, EURORDIS*

HTA and market access

*A. Fehervary, Novartis Farma*

Discussion

Closing remarks: from the “evidence based medicine” to the decision for a single patient  
*Pawel Januszewicz, Polish Medicines Institute*



# Registration Form

Please return the form to GIANNI BENZI PHARMACOLOGICAL RESEARCH FOUNDATION

Tel. +39 080 9643146 • Fax +39 080 9643144 • mail: secretary@benzifoundation.org

Name \_\_\_\_\_ Surname \_\_\_\_\_

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Tel. \_\_\_\_\_ Fax \_\_\_\_\_ E-mail \_\_\_\_\_

Organisation \_\_\_\_\_ Job title \_\_\_\_\_

Address of Organisation \_\_\_\_\_

Post code \_\_\_\_\_ Town \_\_\_\_\_ Country \_\_\_\_\_

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Public Health and University	€ 1.000,00	€ 1.200,00	€ 1.500,00
Graduate Students	€ 500,00	€ 700,00	€ 1.000,00

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**Bank transfer to: Gianni Benzi Foundation**

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**Date**

**Signature**

### PRIVACY

In conformity with the Italian Legislative Decree n. 196, art. 13 of 30 June 2003, the "Gianni Benzi" Foundation informs you that personal data will be handled for organisational purposes and for sending free of charge the documentation related to other congresses or initiatives organised by the Gianni Benzi Foundation only and will be by no means released to third parties. As provided by art. 7 of the Decree n. 196, art. 13 of 30 June 2003, you can contact Gianni Benzi Foundation for further information (crossing out, correction, integration of data). If you agree, please give us your authorization. I authorised the handling of my personal data for the above mentioned aims:

YES ☐

NO ☐

**Date**

**Signature**

## How to reach Krakow

The **John Paul II International Airport Krakow – Balice** and **Airport Pyrzowice** in Katowice are the nearest airport.

11 km to Park Inn Hotel in Krakow from Airport Krakow – Balice ([www.krakowairport.pl](http://www.krakowairport.pl))

100 km to Park Inn Hotel in Krakow from Airport Pyrzowice in Katowice ([www.katowice-airport.com](http://www.katowice-airport.com))

### Arriving at Krakow Balice

Airport Krakow - Balice is connected to Krakow through:

**Access to the airport (more information on website: [www.krakowairport.pl/en/60/4/176](http://www.krakowairport.pl/en/60/4/176) )**

Train - fast railway line to the centre of Krakow - Operated by "PKP Przewozy Regionalne Sp. z o.o.

The train service provided by PKP Przewozy Regionalne Sp. z o.o is the fastest link between the centre of Krakow and the airport. Travel time is 16 minutes. The train station is located 200 m from the T1 international terminal. For travel between the terminals and the station, we advise you to use the free Shuttle Bus service. The Shuttle Bus awaits at the rail station and transports passengers between the international (T1) and the domestic (T2) terminals and then comes back to the station.

Bus Service - Buses of Miejskie Przedsiębiorstwo Komunikacyjna S.A in Krakow (the Municipal Transport Company, MPK S.A.). Krakow Airport is served by two regular bus lines: 208 and 292 and one night line: 902. (These are AGGLOMERATION BUS LINES.

Bus stops within the airport's premises are located near the T1 and T2 terminals.

**Check the location of bus stops on the airport map**

Taxi - **Radio Taxi 19191 - Official partner of Krakow Airport.** RADIO TAXI 19191 provides a 24 hour individual transportation service. Taxi ranks are located in front of the T1 and T2 terminals exits. See taxi ranks on the airport map:

To get a cab, call: + 48 12 19191 - + 48 800 19 19 19

### Arriving at Pyrzowice in Katowice

- Mini-buses and buses run daily business AWOS route Pyrzowice - Krakow – Pyrzowice [www.awos.com.pl](http://www.awos.com.pl)

- Minibus Company Carpolonia run on routes from the airport to Krakow [www.carpolonia.pl](http://www.carpolonia.pl)

- Matuszek Buses run on routes: Krakow – Katowice [www.matuszek.com.pl](http://www.matuszek.com.pl)

## Course Location

### **PARK INN BY RADISSON 4\***

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# Speakers List

Robert Ancuceanu

*Member, Committee for Medicinal Products for Human Use (CHMP) and Member, Paediatric Committee (PDCO), European Medicines Agency (EMA) and Chief of European Integration Service, National Medicines Agency, Romania*

Paola Baiardi

*Director, Consortium for Biological and Pharmacological Evaluations (CVBF), Italy*

Enrico Bosone

*SIAR representative in the "Gianni Benzi" Foundation Board and Regulatory Affairs and Compliance Director, Celgene, Italy*

Daniel Brasseur

*Chair, Paediatric Committee (PDCO) European Medicines Agency (EMA)*

Giuseppe Caruso

*Director Scientific and Innovation Area, Farminindustria, Italy*

Mariana Catapano

*Director, Italian Group for Pharmacoeconomic Studies (GISF), Italy*

Adriana Ceci

*President, "Gianni Benzi" Pharmacological Research Foundation, Italy and Paediatric Committee (PDCO) European Medicines Agency (EMA)*

Anna Cieslik

*Head of Documentation Assessment Department, National Medicines Institute, Poland*

Kevin Connolly

*Member, Paediatric Committee (PDCO) European Medicines Agency and Portiuncula Hospital, Ireland*

Luca De Nigro

*P.M. Monitorised Drugs Registers, Italian Medicines Agency*

Oscar Della Pasqua

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

Bozenna Dembowska-Baginska

*Member, Committee for Orphan Medicinal Products (COMP), European Medicines Agency (EMA) and Instytut Pomnik Centrum Zdrowia Dziecka, Poland*

Fergal Donnelly

*Directorate-General for Research and Technical Development, European Commission*

Catarina Edsjæll

*Senior Director, Head of Regulatory Strategy Europe, Celgene International, Switzerland*

Andras Fehervary

*Director Market Access, Business Unit Oncologia, Regione Europe, Novartis Farma*

Jorge Gallego

*INSERM, Hopital Robert Debré, France*

Jacek St. Gralinski

*Clinical Executive, The Children's Memorial Health Institute, Warsaw, Poland*

Agnes Gyurasics

*Member, Committee for Medicinal Products for Human Use (CHMP) and PDCO member, European Medicines Agency (EMA); Chief Advisor to the Director General, National Institute of Pharmacy, Hungary*

Tim Higenbottam

*Director of Corporate Clinical Development, Chiesi Farmaceutici*

# Speakers List

François Houyez  
*Health Policy Director, EURORDIS - European Rare Diseases Organisation, France*

Ian Hudson  
*Director of Licensing Division, Medicines Control Agency and CHMP member EMA*

Massimo Iacobelli  
*Scientific Director, Gentium Spa, Italy*

Tomasz Jablonski  
*Legal Administrator, Legal Service –Directorate, European Medicines Agency*

Pawel Januszewicz  
*Head of the Paediatric Pharmacology Unit, National Medicines Institute, Poland*

Lucia Lambiase  
*Head of Drug Regulatory Affairs, Novartis Farma, Italy*

Marco Marchetti  
*Director HTA Unit, University Hospital "Agostino Gemelli", Università del Sacro Cuore, Italy*

Boris Matrot  
*INSERM, Hopital Robert Debré, France*

Annarita Meneguz  
*Head of Unit of Biochemical Pharmacology and Technical Scientific Advice Coordination Unit, Department of Drug Research and Evaluation, National Institute of Health, Italy*

Dirk Mentzer  
*Pharmacovigilance, Head of Unit, Paul-Ehrlich-Institut (PEI), Langen, Germany and Paediatric Committee (PDCO) European Medicines Agency (EMA)*

Marek Migdal  
*PDCO member, European Medicines Agency (EMA) and Deputy Head of PICU, Children's*

*Memorial Health Institute, Poland*

Paolo Paolucci  
*PDCO member, European Medicines Agency (EMA) and Professor of Paediatrics and Head Department, University of Modena and Reggio Emilia, Italy*

Simona Paratore  
*CML Franchise Medical Adviser, Novartis Farma, Italy*

Michal Pirozynski  
*Professor of Medicine, Department of Anesthesiology and Critical Care Medicine, Postgraduate Medical School, Poland; former CHMP member EMA*

Agnés Saint-Raymond  
*Head of Sector, Human Medicines Special Areas, European Medicines Agency (EMA)*

Paolo Siviero  
*Drug Strategy and Policy Coordinator Area, Italian Medicinal Agency*

Miriam Sturkenboom  
*Professor of Pharmaco-epidemiology, Erasmus University Medical Center*

Olof Tyden  
*EUREDA - Strategic Consulting for the International Pharmaceutical Industry, Sweden*

Ferdinando Vegni  
*Executive Director - Europe & Asia Pacific Global Drug Safety & Risk Management, Celgene International Sàrl, Switzerland*

Chris Walker  
*Director for International Regulatory Affairs, Amgen, UK*

Piotr Zukowski  
*Medical Director, Sanofi-Aventis, Poland*

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