4th Foresight Training Course

Evidences for Rational Therapies: From Newborn to Elderly Population

1-3 September • Rome, Italy

Gianni Benzi Pharmacological Research Foundation

Dear Colleagues,

for the 4th Foresight Training Course we chose Rome, where the ancient and the modern meet in every field.

This year the Course also applies for the Italian system of educational credits (ECM) for healthcare professionals.

The Gianni Benzi Foundation is, in fact, authorised to assign ECM credits according to the latest Italian law (provider n° 1595) for on site educational activities and e-learning courses.

The Foresight Training Courses are international events addressed to Medical Directors, R&D Managers, Pharmacovigilance Managers, Clinical Research Associates, Clinical Project Managers/ Leaders, Regulatory Affairs Operators, Market Access Operators, Statisticians, Information Technology Operators and Data Managers of Pharmaceutical Industries, but also to Managing Directors and Researchers (Medical and not-medical) of Research Centers, Professors and Students, Business Development Managers, Medical Directors, Project Managers, Clinical Research Associates working in CROs, and finally Managing Directors working in the Biotech field. The Courses are aimed to underline the more recent and stimulating debates open in the field of the European Pharmaceutical System and to contribute actively to them.

In line with the previous editions, the Course is inspired by the European trends concerning pharmaceuticals, with special attention to the advancement in the Clinical Trials methodology and other studies supporting drug development and post-marketing drugs use.

The current debate on how to support and consolidate clinical evidences in different settings and different diseases by collecting useful data from the drug developmental phase to post-marketing use, will be addressed having as example the working methodology already validated by EMA and other National Authorities.

As other editions, the Course gains its strength by the involvement of all the interested stakeholders (Regulators, Researchers, Physicians, Health Professionals, Private and Public Companies, Patient Associations) and by the collaboration with such organisations as the Children's Memorial Health Institute of Warsaw, the National Center for Biological Research (CNRB), the Middle European Association for Regulatory Affairs (MEGRA), the Italian Society of Regulatory Activities (SIAR) and the Task Force in Europe for Drug Development in the Young (TEDDY). Farmindustria, the Italian pharmaceutical industry association, contributed also to the realisation of this Course with an educational grant.

We sincerely hope that you will be able to participate in the Course and enjoy contents, discussions and social dinners.

With our best wishes,

Adriana Ceci

'Gianni Benzi' Foundation
President

Enrico Bosone

'Gianni Benzi' Foundation
SIAR Representative in the Board

1-3 SEPTEMBER 2011 • ROME • ITALY

4th FORESIGHT TRAINING COURSE

EVIDENCES FOR RATIONAL THERAPIES: FROM NEWBORN TO ELDERLY POPULATION

organised by

the 'Gianni Benzi' Pharmacological Research Foundation

Scientific collaboration:

- **Children's Memorial Health Institute of Warsaw**
- **CNRB - National Center for Biological Resources**
- **MEGRA - Middle European Association for Regulatory Affairs**
- **SIAR - Italian Society of Regulatory Activities**
- **TEDDY - Task Force in Europe for Drug Development in the Young**

Educational Grant from FARMINDUSTRIA

BACKGROUND

This fourth Course of the Gianni Benzi Foundation is inspired by the European Programs concerning Health, with special attention to the EMA Road-Map 2010-2015, and to the national Health Plans (such as the Italian oncologic plan 2011-2013). In fact the European activities take into account the national issues and, on the other hand, the local level is influenced by the outcomes of the European Process.

Health Authorities' decisions are based on the "regulatory sciences" that include the **scientific evidences** provided both by "basic and applied medicinal science", continuously updated. Up to now scientific evidences are collected mainly during the drug developmental process, in particular through the pre-marketing registrative clinical/non-clinical trials. However the rules and methodologies to assess scientific evidences could vary in accordance with relevant patients characteristics - such as age (from birth to elderly), sex, geographic and social features, etc. - or disease characteristics (chronic versus acute, infective versus degenerative, immunological, oncological, etc.), as well as product characteristics (biological versus chemical, AT, etc.).

In addition, when relevant unmet medical needs are concerned and positive preliminary data are available, timely management of regulatory decisions is of paramount importance. For that reason, allowing access to therapies without having yet the complete knowledge of the consequences of the therapy can be acceptable in some cases.

In these cases the subsequent collection of clinical evidences, supplied by phase IV CTs, observational studies, active and passive pharmacovigilance, Registries of therapies and/or diseases, can help in the definition of the best therapeutic strategy.

Three strategic areas are depicted in the EMA Road-Map: the **unmet public-health needs**, **access to therapies** and **rational use of therapies**. They can be taken as a common denominator of all the strategies aiming to improve the Health in each Member State, both in Europe and in the World.

The oncology field represents a significant example in this sense (see the Italian national oncologic plan 2011-2013 as an example) since all these approaches are applied and all these efforts are translated in dedicated plans and guides covering again 'by birth to elderly'.

The fourth Course of Gianni Benzi Foundation wishes to start from this picture having the objective to examine the different phases of R&D for new therapies, to underline the opportunities made available by the existing collaborative international networks, to stimulate additional collaborative efforts having as example the working methodology already validated by EMA and other national Authorities like AIFA. Special attention will be focused on the advancement in the Clinical Trials methodology and other studies supporting drug development and post-marketing drug uses (revision of CT Directive, Pharmacovigilance implementation, EudraCT and so on).

COURSE DIRECTORS

Enrico Bosone	SIAR representative in the Gianni Benzi Foundation Managing Board; Regulatory and Public Affairs Director, Celgene srl
Marek Migdal	Deputy Head of PICU, Children's Memorial Health Institute, Warsaw, and member of Paediatric Committee (PDCO), EMA
Paola Baiardi	CVBF representative in the Gianni Benzi Foundation Board and member of the Paediatric Working Group-AIFA
Mariana Catapano	GISF Director and Regulatory Science Expert

Organising Secretariat
Rossella Conte
secretary@benzifoundation.org

PROGRAM

01.09.2011

Welcome Session
in the presence of the Authorities

Strategic areas for therapies in Europe: Health Authorities, Academy, Industry facing unmet medical needs (9:30 am – 1:00 pm)

Chairs: Vittorio Silano - Walter Bianchi

Introduction to the Course	A. Ceci - E. Bosone
EMA Road Map perspectives and present status	Vincenzo Salvatore
How the regulatory system can be attuned to science	Ian Hudson
How to address orphan therapeutic needs	Kerstin Westermark
How to integrate research needs and children protection	Daniel Brasseur
How Science must reduce human sufferings	Pawel Januszewicz
Role of Industry	Farmindustria
Role of research networks	Carlo Giaquinto

Discussion

1:00 pm Lunch

Session I - How to provide scientific evidences: different approach for different settings (2:00 pm - 6:00 pm)

Patients population

Chairs : Jerry Zeldis - Domenico Criscuolo

PIPs and paediatric population: current status	Gunter Egger
Neonatal and paediatric intensive care	Marek Migdal
CTs designed for old people	Simonetta Alvino
CTs in fertile pregnant women and adolescents	Viveca Odlind
Appropriate formulations: from neonates to old patients	Siri Wang

Discussion

4:00 pm Coffee-break

Specific diseases

Chairs: Walter Bianchi-

Haematology-oncology	Daniele Alberti
Paediatric oncology	Paolo Paolucci
Immunology	Paolo Rossi
Rare diseases	Carlos Camozzi
Neglected diseases	Jerry Zeldis

Discussion

6:00 pm Rome tour and dinner

02.09.2011

Round Table 1 - The future European Union Legislation for CT (8:30 am - 11:00 am)

Chairs: Daniel Brasseur - Leonardo Santi

Presentations:

Carlo Tomino
Which role for EUDRACT to cover unmet needs?

Ian Hudson
Are the Clinical Trials and the GCP Directives to be changed?

Discussants:

COMP (Committee for Orphan Medicinal Products - EMA)	Kerstin Westermark
EUCROF (European CRO Federation)	Martine Dehlinger-Kremer
CNRB (National Center for Biological Research)	Dino Amadori
EMA (European Medicines Agency)	Gunter Egger
GRIP (Global Research in Paediatrics) & TEDDY	Adriana Ceci
EURORDIS (Rare Diseases in Europe)	Dorica Dan
INDUSTRY	Giuseppe Caruso
	Fabien Peuvrelle

Discussion

11:00 am Coffee-break

Session II - Behind Registrative Clinical Trials: how to use different tools to increase clinical evidences (11:15 am - 1:00 pm)

Chair: Enrico Bosone

The Registrative use of non-interventional and/or non sponsored studies: is it allowed? How to regulate it?	Carlo Tomino
Health Records databases, pharmacoepidemiology and drug development	Miriam Sturkenboom
Rare diseases registries as a tool for patients	Domenica Taruscio
Non conventional studies and orphan drug evaluation	Paola Baiardi
Observational studies inspired by Treatments' Registries	Antonio Del Santo
PAH in children	Malgorzata Zuk

Discussion

1:00 pm Lunch

Session III – Behind the drug development: what is at the door? (2:00 pm – 3:30 pm)

Chair: Rodolfo Paoletti

Introduction: *Menotti Calvani*

Generics as a tool	Pia Furlani
Nutraceuticals: which role for human health?	Andrea Poli
Are Biosimilar a new or an old drug?	Chris Walker
The role of Biotechnologies	Domenico Criscuolo

Discussion

3:30 pm Coffee-break

Session IV – Rational use of Medicines: Pharmacovigilance in the light of the forthcoming legislation (3:45 pm – 6:00 pm)

Chairs: Miriam Sturkenboom

Legal aspects and implementation at EMA level	Vincenzo Salvatore
Implementation at national level	Fernanda Ferrazin
Active pharmacovigilance in paediatrics	Antje Neubert
Pharmacovigilance and market access	Paolo Biffignandi
Signaling from non-clinical studies	Annarita Meneguz

Discussion

6:00 pm Rome tour and dinner

03.09.2011

Session V – Access to therapies for patients and health professionals

The true innovation is the improvement of the clinical benefit (8:30 am – 11:15 am)

Chair: Renza Galluppi

Access to therapy in rare conditions	Dorica Dan
Met and unmet medical needs for old people	Agnes Gyurasics
Women and therapeutic needs	Flavia Franconi
Met and unmet medical needs in haematology	Robin Foà
Met and unmet medical needs in neurology	Luca Massacesi
Met and unmet medical needs in oncology	Paolo Marchetti
Needs in paediatric oncology	Bozenna Dembowska-Baginska

Discussion

Round Table II – Models for the assessment of B/R ratio, Comparative Benefit/Risk ratio, cost-benefit and HTA experiences at international (EUnetHTA) and national levels (11:15 am – 12:30)

Chair: Walter Bianchi

Presentation:

Jacek Gralinski

Public Health needs at national level

Marco Marchetti

HTA experiences at international and national level

Discussants:

Anna Cieslik

Olof Tyden

Enrico Bosone

Discussion

12:30 Closing Remarks: Course Directors

1:00 pm Light Lunch

SPEAKERS LIST

SURNAME	NAME	AFFILIATION
Alberti	Daniele	Novartis Farma S.p.A., Oncology Medical Director, Italy
Alvino	Simonetta	Pharmanet, Medical Director, Italy
Amadori	Dino	Institute for the Study and Treatment of Cancer (Forlì), Scientific Director, Italy
Baiardi	Paola	Consortium for Biological and Pharmacological Evaluations, Director, Italy
Biffignandi	Paolo	VI.REL Pharma S.a.s. - Regulatory Affairs Consultancy, Director, Italy
Bianchi	Walter	Italian Society of Regulatory Affairs, President, Italy
Bosone	Enrico	'G. Benzi' Pharmacological Research Foundation, Italian Society of Regulatory Affairs Representative in the Board, Italy
Brasseur	Daniel	European Medicines Agency, Chair Paediatric Committee, Belgium
Calvani	Menotti	Fondazione SigmaTau, Vice-President, Italy
Camozzi	Carlos	AMT Biopharmaceuticals, Chief Medical Officer, France
Caruso	Giuseppe	Farmindustria, Scientific and Innovation Area Director, Italy
Catapano	Mariana	Italian Group for Pharmacoeconomics Studies, Director, Italy
Ceci	Adriana	'G. Benzi' Pharmacological Research Foundation, President, Italy
Cieslik	Anna	Polish National Medicines Institute, Head Documentation Assessment Department, Poland
Criscuolo	Domenico	Genovax, Chief Executive Officer, Italy
Dan	Dorica	EURORDIS, Board Member, Romania
Del Santo	Antonio	Roche S.p.A. Italy, Medical Director, Italy
Dembowska-Baginska	Bozenna	European Medicines Agency, Committee for Orphan Medicinal Products Member, Poland
Dehlinger-Kremer	Martine	Theorem Clinical Research, Vice President, Germany
Egger	Gunter	European Medicines Agency, Scientific Administrator, UK
Ferrazin	Fernanda	Italian Medicines Agency, Head of the Post-Marketing Surveillance Area, Italy
Foà	Robin	'Sapienza' University of Rome, Head of Hematology Division, Italy
Franconi	Flavia	Italian Society of Pharmacology, Gender-oriented Group Coordinator, Italy
Furlani	Pia	DOC Generici srl, Regulatory Affairs Director, Italy
Galluppi	Renza	UNIAMO, Italian Federation Rare Diseases Onlus, President, Italy
Giaquinto	Carlo	GRIP and PENTA Networks Coordinator, Italy
Gralinski	Jacek	The Children's Memorial Health Institute, Clinical Director, Poland
Gyurasics	Agnes	European Medicines Agency, Committee for Medicinal Products for Human Use Member, Hungary

SURNAME	NAME	AFFILIATION
Hudson	Ian	European Medicines Agency, Committee for Medicinal Products for Human Use Member, UK
Januszewicz	Pawel	Polish National Medicines Institute, Head of Paediatric Pharmacology Unit, Poland
Marchetti	Marco	University Hospital 'Agostino Gemelli', Director HTA Unit, Italy
Marchetti	Paolo	University Hospital 'Sant'Andrea', Head of Oncology Unit, Italy
Massacesi	Luca	Faculty of Medicine, Professor of Neurology, Department of Neurological and Psychiatric Sciences, Italy
Meneguz	Annarita	Italian National Institute of Health, Head of Unit of Biochemical Pharmacology and Technical Scientific Advice, Italy
Migdal	Marek	European Medicines Agency, Paediatric Committee Member, Poland
Neubert	Antje	University Hospital Erlangen, Head of Paediatric Clinical Study Center, Germany
Odlind	Viveca	European Medicines Agency, Paediatric Committee, Member, Sweden
Paoletti	Rodolfo	Nutrition Foundation of Italy, President, Italy
Paolucci	Paolo	European Medicines Agency, Paediatric Committee, Member, Italy
Peuvrelle	Fabien	Celgene R&D Sàrl, Regulatory Operations Europe Regulatory Affairs Director, Switzerland
Poli	Andrea	Nutrition Foundation of Italy, Scientific Director, Italy
Rossi	Paolo	European Medicines Agency, Paediatric Committee Member, Italy
Salvatore	Vincenzo	European Medicines Agency, Head of Legal Sector, UK
Santi	Leonardo	National Center for Biological Resources, president, Italy
Silano	Vittorio	'G. Benzi' Pharmacological Research Foundation, Scientific Commission President, Italy
Sturkenboom	Miriam	Erasmus University Medical Center, Professor of Pharmaco-epidemiology, The Netherlands
Taruscio	Domenica	Italian National Institute of Health, National Centre for Rare Diseases Director, Italy
Tyden	Olof	EUREDA, Strategic Consulting International Pharmaceutical Industry, Sweden
Tomino	Carlo	National Monitoring Centre on Clinical Research with Medicines (OsSC), Director, Italy
Wang	Siri	European Medicines Agency, Head of PDCO Formulation Subgroup, Norway
Walker	Chris	Amgen, Director for International Regulatory Affairs, UK
Westermark	Kerstin	European Medicines Agency, Committee for Orphan Medicinal Products President, Sweden
Zeldis	Jerry	Celgene Global Health and Celgene Corporation, CEO and Chief Medical Officer, USA
Zuk	Malgorzata	Children's Memorial Health Institute, Head of Cardiology Department, Poland

Please return the form to GIANNI BENZI PHARMACOLOGICAL RESEARCH FOUNDATION
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	Full Course	Full Course + Dinners	Full Course + Dinners + Hotel (2 nights)
Standard	€ 2.250	€ 2.400	€ 2.700
Graduate Students	€ 600	€ 750	€ 1.050

Method of Payment

Bank transfer to: Gianni Benzi Foundation
IBAN code: IT 63 I 0101041750100000001097 – BIC / SWIFT code: IBSPITNA

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 the personal data will be handled for organizational purposes and for sending free of charges the documentation related to other congresses or initiatives organized 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 authorized the handling of my personal data for the above mentioned aims:

○ YES ○ NO

Date _____ Signature _____

How to reach Rome

Leonardo da Vinci (Fiumicino Rome) and G. B. Pastine (Ciampino Rome)
are the airports of Rome
www.adr.it

There are several bus services that link Rome to its airports
www.sitbusshuttle.it

The main railway station of Rome is Termini
The Underground system is connected to the railway station, and there are others railway connections between Airports and Termini station
www.trenitalia.com

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Course Location

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Aran Mantegna Hotel's front entrance – Rome


A view of the Coliseum - Rome

Organised by

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FOUNDATION

In collaboration with

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www.cnrb.it

Instytut "Pomnik - Centrum Zdrowia Dziecka"
The Children's Memorial Health Institute
www.czd.waw.pl

MEGRA
Middle European Association for Regulatory Affairs

SIAR
Società Italiana Attività Regolatorie

TEDDY
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www.teddyoung.org

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