



**GIANNI BENZI**

PHARMACOLOGICAL RESEARCH  
FOUNDATION

EUROPEAN  
BIOTECH  
WEEK



*Distretto H-BIO Puglia*

6<sup>th</sup> **“BIOTECH AND  
Foresight  
Training  
Course** **INNOVATIVE SCIENCE  
TO MEET PATIENT NEEDS”**  
Bari, 30<sup>th</sup> September - 2<sup>nd</sup> October 2013



### Key concepts and Course presentation

“Healthcare biotech is already benefiting more than 350 million patients around the world through the use of biotech medicine to treat and prevent every day acute and chronic illnesses including heart attacks, stroke, multiple sclerosis, breast cancer, cystic fibrosis, leukaemia, diabetes, hepatitis, etc” (Beyond borders, 2012).

Nevertheless, as stressed in the European Medicines Agency (EMA) Road Map to 2015, “an important public-health challenge currently faced is the lack of medicines for unmet medical needs”. For example, today in Europe, despite the evident need, there is still a dramatic lack of treatments for 30 million Europeans affected by any of the 5,000 to 7,000 rare diseases. In addition, special population including elderly and children have few therapeutic options tailored for their medical unmet needs.

This year the annual Foresight Training Course of the Gianni Benzi Pharmacological Research Foundation will take its cue from the advancement in biotechnology to address patient needs and is designed around the following topics:

- Role and Relevance of Biotechnology in the process of Drug Innovation
- Biotechnology covering therapeutic needs
- Regulatory Controversies and Novelties

The Course will be the occasion to discuss biotech results and challenges by different stakeholders points of view: in particular the Course will focus on the promises of the market and the relevance of the partnering of small companies with Big Pharma, on the effects (risk and benefits) of the ongoing regulatory initiatives in the pharmaceutical sector, on patients expectancies and, overall, on the acquired and on-going scientific results.

**THE COURSE IS ORGANISED IN THE CONTEXT OF THE:**



The Course will provide 22,2 Italian ECM credits for Healthcare Professionals, and will be held in Bari, on the initiative of the Gianni Benzi Pharmacological Research Foundation and the High-Tech District Biotechnology and Health (H-BIO Puglia).

## **COURSE PROMOTERS**

### **Gianni Benzi Pharmacological Research Foundation**

The Gianni Benzi Pharmacological Research Foundation is a registered “not-for-profit” Scientific Research Organisation, founded on 9 November, 2007. The mission is to contribute to the advancement of pharmacological research in those fields where the scientific heritage of Prof Gianmartino Benzi has been more prominent thus focusing on Innovative Medicines, medicines for rare or neglecting diseases and medicines for ‘special’ populations including children.

While supporting pharmacological research, the Foundation is especially committed to provide educational support and to set up activities in the Regulatory Science sector having as reference the European dimension as the natural framework to favoring innovation, to defining common rules and adequate procedures, to ensuring equality of access to efficacious and safe tools and medicinals to all European citizens. The Foresight Training Courses, annually organized by the Gianni Benzi Pharmacological Research Foundation are short courses for researchers and experts in the field of Regulatory Sciences that aim at contributing to satisfy the increasing knowledge needs of the sector.

### **High-Tech District Biotechnology and Health (H-BIO Puglia)**

H-Bio Puglia is a High Technology District in the field of health and biotechnology with the aim of enhancing innovation in the manufacturing system in the Italian Region of Apulia. The activities will be implemented by an important network involving universities, research centres and biotech companies, with the intention to respond to fundamental needs: on the one hand the need of the citizens to safeguard their right to ever more advanced and innovative therapies, and on the other hand the need of research and development that try to meet this demand.

The main areas of interest of the District are represented by the development of personalised medicine (mainly based on biomarkers derived by the DNA/RNA technologies), advanced therapies (including gene therapy, cellular and tissue therapy), innovative diagnostics (including drug discovery and drugs delivery) and new manufacturing processes.

The High-Tech District H-BIO Puglia was established as Consortium on September 14th 2012 and involves a total of 36 public and private partners.



## COURSE COORDINATORS

**Adriana Ceci**

Gianni Benzi  
Pharmacological Research Foundation  
President

**Maria Svelto**

H-BIO  
High Technology District  
President

## COURSE SCIENTIFIC COMMITTEE

**Enrico Bosone** - SIAR, Italian Society for Regulatory Activities (Italy)

**Vito Michele Fazio** - IRCCS Ospedale Casa Sollievo della Sofferenza (Italy)

**Viviana Giannuzzi** - Gianni Benzi Pharmacological Research Foundation (Italy)

**Marek Migdal** - Children's Memorial Health Institute Warsaw (Poland)

**Vincenzo Salvatore** - University of Insubria (Italy)

## ORGANISING SECRETARIAT

Gianni Benzi Pharmacological Research Foundation

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# “BIOTECH AND INNOVATIVE SCIENCE TO MEET PATIENT NEEDS”

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9,30 am

## WELCOME SESSION

### *Introductory remarks*

Prof. Vittorio Silano,  
Gianni Benzi Foundation Scientific Committee, President

### *Institutional representatives*

Prof. Giuseppe Speciale, Confindustria Puglia  
Prof. Francesco Losurdo, Comunità delle Università del Mediterraneo  
Dott. Ludovico Abbaticchio, Assessore al Welfare, Comune di Bari  
Dr. Anna Cieslik, Director, Office for Registration of Medicinal Products, Poland  
Dott.ssa Elena Gentile, Assessore alla Salute e al Welfare, Regione Puglia  
Prof. Corrado Petrocelli, Rettore dell'Università degli Studi di Bari

## INTRODUCTORY SESSION

Introduction of the Course  
(A. Ceci, Gianni Benzi Foundation President)  
The H-BIO District: challenges and opportunities  
(M. Svelto, H-Bio Puglia President)  
Regulatory Science relevance and Biotechnology  
(W. Bianchi, SIAR President)  
Innovation in the Pharmaceutical field  
(A. G. Del Santo, Farindustria-Biotechnology Group)

11,00 am

## I SESSION

### Role and Relevance of Biotechnology in the process of Drug Innovation

**Chairs of the session: M. Svelto - A. Ceci**

EATRIS, the European Infrastructure for translational research in Europe  
(G. Migliaccio, EATRIS)  
Trends in the European Biotechnology Market  
(A. Irione, EY)  
Innovative Medicines Initiative, the largest public-private initiative to boost pharmaceutical innovation in Europe  
(G. Ruberti, CNR)  
High Technology Districts and Clusters in the national and international framework  
(F. Conicella, Bioindustry Park)  
Big Pharma, SMEs and Academy collaboration to foster Biotechnology innovation  
(M. L. Nolli, Assobiotech)

2,00 pm

## II SESSION

### Biotechnology to cover therapeutic needs

**Chairs of the session: M. Saviano - S. Cotecchia**

Towards the Personalised Medicines: microRNA targeting Neuroblastoma  
(M. Saviano, CNR-Institute of Crystallography)

Experimental and computational strategies for large scale investigation  
of human mutations involved in genetic diseases through whole exome sequencing:  
implications for early diagnosis and personalized therapies  
(G. Pesole, CNR - Institute of Biomembranes and Bioenergetics)

Therapeutic vaccines  
(D. Criscuolo, Genovax)

Partnerships in biotechnology: what Big Pharmas want – the vaccine prospective  
(L. Toller, Novartis Vaccines and Diagnostics)

Molecular mechanisms of BMF in paroxysmal nocturnal haemoglobinuria (PNH)  
(L. Luzzatto, Istituto Toscano Tumori)

4,30 pm

## III SESSION

### Biotechnology in oncology

**Chairs of the session: V. Fazio - G. Migliaccio**

Advancement in biotechnological research in oncology  
(V. M. Fazio, IRCCS Ospedale Casa Sollievo della Sofferenza)

Genomic approach to discover human cancer pathways  
(M.T. Landi, NIH USA)

Cancer Stem Cells  
(A. Vescovi, IRCCS Ospedale Casa Sollievo della Sofferenza)

Prognostic microRNA/mRNA signature and personalised medicine  
(S. Volinia, University of Ferrara)

Biomarkers and Companion Diagnostic Tests in targeted Drug Development  
(A. Mathieu-Boue, Novartis Oncology)

Engineering Monoclonal Antibodies for cancer therapy  
(M. Allegretti, Dompè)

# “BIOTECH AND INNOVATIVE SCIENCE TO MEET PATIENT NEEDS”

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9,00 am

## IV SESSION

### Innovation in Paediatrics and Therapeutic Needs

**Chairs of the Session: A. Costescu - P. Rossi**

The EU promotes paediatric research  
(A. Costescu, EU Commission)

Translating research in drugs development: the lesson  
from the EU funded paediatric research  
(A. Ceci, Gianni Benzi Foundation; PDCO-EMA)

Therapeutic Needs and Paediatric trial  
(M. Catapano, Italian Group for Pharmacoeconomic Studies)

NeoMero - European multicenter network to evaluate PK, safety and efficacy of Meropenem in  
neonatal sepsis and meningitis  
(I. Lutsar, University of Tartu, Estonia; PDCO-EMA)

DEEP - DEferiprone Evaluation in Paediatrics  
(D. Bonifazi, Consortium for Biological and Pharmacological Evaluation)

11,00 am

## V SESSION

### Paediatric Networks to improve research

**Chairs of the session: M. Migdal - V. Giannuzzi**

The Paediatric Networks improving paediatric research  
(C. Giaquinto, GRiP Network Coordinator, TEDDY Network Co-Coordinator)

InNerMeD-I-Network - Inherited NeuRoMetabolic Diseases Information Network  
(F. Bonifazi, Gianni Benzi Foundation)

Paediatric Needs – are we going the right direction?  
(A. Neubert, University Hospital Erlangen)

Stem cells use in children  
(B. Dembowska-Baginska, COMP-EMA)

How to involve children in the regulatory decisions  
(E. Davies, Elin Haf Davies Enabling Research, Empowering Children)

2,30 pm

## VI SESSION

### Biotechnology and coagulopathies

**Chairs of the session: P. Giordano - E. Bosone**

Recent progress in the field of coagulopathies  
(P. Giordano, University Bari 'Aldo Moro')

Advances in haemophilia  
(P. Mathew, Bayer HealthCare)

New trends in children anticoagulation  
(A. C. Molinari, IRCCS G. Gaslini)

Expanding the Boundaries of Haemophilia Care: new drugs in development  
(S. Seremetis, Novo Nordisk)

Gene therapy in haematological disorders  
(S. Acuto, AOR Villa Sofia-Cervello/P. Cutino Foundation)

4,30 pm

## VII SESSION

### Aged people and therapeutic Innovation

**Chairs of the Session: M. Scatigna - R. Bernabei**

Therapeutic needs in ageing : the EMA geriatric medicines strategy  
(N. Marchionni, EMA GEG, University of Firenze)

The CHMP role for a sustainable drug development for old people  
(A. Gyurasics, CHMP-PDCO)

Ongoing pharmacological research and expected results  
(A. Genazzani, University 'Piemonte Orientale')

Frailty syndrome and dementia in aged people  
(G. Logroscino, University of Bari 'Aldo Moro')



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**9,00 am**

**VIII SESSION**

**Outstanding regulatory and legislative issues -1**

**Chairs of the Session: V. Salvatore - A. Natz**

How to deal with regulatory controversies: the V Foresight Course Lesson  
(E. Bosone, Celgene)

GCP: a challenge for Biotech studies and Research  
(A. Del Vecchio, AIFA)

The new Regulation on Clinical Trials  
(A. Altavilla, Espace Ethique Méditerranéen)

A paediatric update: the 5-year report and the public consultation  
(M. Migdal, PDCA- EMA)

Clinical Trials: the Transparency of results and the access to data  
(V. Salvatore, University of Insubria)

**11,30 am**

**IX SESSION**

**Outstanding regulatory and legislative issues -2**

**Chairs of the Session: G. Banfi - A. Del Vecchio**

Price Regulation in Europe  
(A. Natz, EUCOPE)

Regulation on Privacy and Data Protection  
(G. Buttarelli, EDPS)

Experts without conflict of interest: an increasing problem?  
(A. Cieslik, Polish NCA)

Medicine supply shortages  
(F. Houyez, EURORDIS)

**Closing Remarks 2,30 pm**

## LIST OF CHAIRS AND SPEAKERS

### **Santina Acuto**

AOR Villa Sofia-Cervello/P. Cutino Foundation (Italy)

### **Marcello Allegretti**

Dompé SpA, Chief Scientific Officer (Italy)

### **Annagrazia Altavilla**

Espace Ethique Méditerranéen - Aix-Marseille University, Associate Senior Lecturer of Bioethics (France)

### **Giuseppe Banfi**

Scientific Director Galeazzi Milan, General Director of the Centro San Raffaele Foundation (Italy)

### **Roberto Bernabei**

University Cattolica del Sacro Cuore, Professor of Internal Medicine; University Hospital A. Gemelli, Department of Geriatrics and Rehabilitative Medicine, Director; Board Member of the European Academy for Medicine of Aging (EAMA).

### **Walter Bianchi**

President of SIAR; Member of the Gianni Benzi Foundation's Board of Directors (Italy)

### **Donato Bonifazi**

Consortium for Biological and Pharmacological Evaluations, Managing Director; DEEP Project Manager (Italy)

### **Fedele Bonifazi**

Gianni Benzi Pharmacological Research Foundation, Vice President; InNerMed-I-Network, Project Manager (Italy)

### **Enrico Bosone**

SIAR Board of Directors Representative (Italy)

### **Giovanni Buttarelli**

European Data Protection Supervisor (EDPS) (Belgium)

### **Mariana Catapano**

Italian Group for Pharmacoeconomics Studies, Director (Italy)

### **Adriana Ceci**

Gianni Benzi Pharmacological Research Foundation, President (Italy); European Medicines Agency (EMA), Member of PDCO

### **Anna Cieslik**

Office for Registration of Medicinal Products, Medical Devices and Biocidal Products, Director of Department of Assessment of Medicinal Products Documentation (Poland)

### **Fabrizio Conicella**

Bioindustry Park Silvano Fumero SpA, Director Business Development and international project; Coordinator at the bioPmed Innovation Cluster (Italy)

### **Alexandru Sorin Costescu**

EU Commission

### **Susanna Cotecchia**

University of Bari "Aldo Moro", Professor of Pharmacology; Consortium for Biological and Pharmacological Evaluations, President (Italy)

### **Domenico Criscuolo**

Genovax S.r.l., Chief Executive Officer (Italy)

### **Elin Haf Davies**

Elin Haf Davies Enabling Research - Empowering Children, Director (UK)

### **Antonio Giuseppe Del Santo**

Farindustria-Biotechnology Group (Italy)

### **Angela Del Vecchio**

Italian Medicines Agency (AIFA), Director Good Clinical Practice and Pharmacovigilance Inspectorate (Italy)

### **Bozena Dembowska-Baginska**

The Children's Memorial Health Institute, Vice Chair, Professor at The Department of Pediatric Oncology; European Medicines Agency (EMA), Member of COMP (Poland)

### **Vito Michele Fazio**

Specialist in Oncology; University Campus Bio-Medico, Area of General Pathology, Chairman (Italy)

### **Armando Genazzani**

University 'Piemonte Orientale', Professor of Pharmacology (Italy)

### **Viviana Giannuzzi**

Gianni Benzi Pharmacological Research Foundation, Head of ethics and education unit (Italy)

### **Carlo Giaquinto**

GRIP and PENTA Networks Coordinator

### **Paola Giordano**

U.O. Pediatria "F. Vecchio" - University Hospital, Policlinic of Bari (Italy)

### **Agnes Gyurasics**

European Medicines Agency (EMA), Member of PDCO and CHMP; National Institute of Pharmacy, Chief Advisor to the General Director (Hungary)

# “BIOTECH AND INNOVATIVE SCIENCE TO MEET PATIENT NEEDS”

## LIST OF CHAIRS AND SPEAKERS

### **Francois Houyez**

European Rare Diseases Organisation (EURORDIS), Health Policy Director (France)

### **Antonio Irione**

EY, Advisory Life Science Leader

### **Maria Teresa Landi**

National Institutes of Health (NIH), Genetic Epidemiology Branch Division of Cancer Epidemiology and Genetics National Cancer Institute, Senior Investigator (USA)

### **Giancarlo Logroscino**

University of Bari ‘Aldo Moro’, Associate Professor of Neurology (Italy)

### **Irja Lutsar**

Professor of Medical Microbiology, University of Tartu (Estonia); European Medicines Agency (EMA), Member of PDCO

### **Lucio Luzzatto**

Scientific Director, Istituto Toscano Tumori, Firenze (Italy)

### **Niccolò Marchionni**

University of Firenze, Professor of Gerontology and Geriatrics; EMA GEG Chair (Italy)

### **Prasad Mathew**

Vice President, Global Medical affairs - Hematology, Bayer Health Care; Professor of Pediatrics, Pediatric Hematology/Oncology University of New Mexico (USA)

### **Anne Mathieu-Boue**

Novartis Oncology, Region Europe, Medical Affairs, Senior Medical Director, Piz Kinase Inhibitors

### **Marek Migdal**

European Medicine Agency (EMA), Member of PDCO; Children’s Memorial Health Institute, Deputy Head, PICU, Head of Long Term Care Unit (Poland)

### **Giovanni Migliaccio**

European Advanced Translational Research Infrastructure in Medicine (EATRIS), Scientific Director (The Netherlands); Italian Institute of Health (ISS), Head of the Gene and Cell Therapy Section (Italy)

### **Angelo Claudio Molinari**

Giannina Gaslini Children’s Hospital, Thrombosis and Hemostasis Unit and Hemophilia Treating Centre, Director (Italy)

### **Alexander Natz**

European Confederation of Pharmaceutical Entrepreneurs (EUCOPE), Secretary-General

### **Antje Neubert**

University Hospital Erlangen, Department of Paediatric and Adolescents Medicine, Head Paediatric Clinical Study Centre (Germany)

### **Maria Luisa Nolli**

Assobiotec, Member of the Executive Committee; Member of the EuropaBio Board; Areta International, Founder and Chief Executive Officer (Italy)

### **Graziano Pesole**

Director of the Institute of Biomembranes and Bioenergetics (IBBE)-CNR; Department of Biochemistry and Molecular Biology “E. Quagliariello”, University of Bari “A. Moro” (Italy)

### **Paolo Rossi**

Children’s Hospital Bambino Gesù, Department of Paediatrics, Chairman (Italy); European Medicine Agency (EMA), Member of PDCO

### **Giovina Ruberti**

National Research Council (CNR), Research Director, Institute of Cell Biology and Neurobiology MIUR Delegate IMI-JU

### **Vincenzo Salvatore**

University of Insubria Varese, Professor of International Law (Italy)

### **Michele Saviano**

Director Institute of Crystallography (IC)-CNR, President of Italian Crystallographic Association (AIC) (Italy)

### **Marco Scatigna**

Sanofi-Aventis, Medical and Scientific Director, General Manager S. A. Foundation (Italy)

### **Stephanie Valerie Seremetis**

Novo Nordisk, Corporate Vice President and Chief Medical Officer Hemophilia R&D Portfolio (USA)

### **Vittorio Silano**

University of Rome II; Chair ESCO Working Group, EFSA European Food Safety Authority; Chair of the Gianni Benzi Foundation’s Scientific Committee

### **Maria Svelto**

High Technology District H-Bio Puglia, President (Italy)

### **Lorenzo Tollerì**

Novartis Vaccines and Diagnostics, Business Development Senior Manager (Italy)

### **Angelo Luigi Vescovi**

IRCCS Casa Sollievo della Sofferenza, Scientific Director (Italy)

### **Stefano Volinia**

University of Ferrara, Department of Morphology and Embryology (Italy)



# Foresight Training Courses

**137 SPEAKERS FROM ALL AROUND THE WORLD**  
(INVOLVING NON-PROFIT ORGANISATIONS, UNIVERSITIES/PUBLIC HEALTH CENTERS, REGULATORY AGENCIES AND INDUSTRY)

**OVER 100 HOURS OF COURSES**

**OVER 350 PARTICIPANTS FROM 21 COUNTRIES**

**50 RESEARCH GRANTS ASSIGNED**



**TOPIC: EUROPEAN CENTRALISED  
PROCEDURE AND PAEDIATRIC  
REGULATION**

**TOPIC: ADVANCED THERAPY  
AND ORPHAN DRUGS**



**PAVIA  
2009**

**KRAKOW  
2010**

**TOPIC: BENEFIT/RISK ASSESSMENT  
OF MEDICINES**



**TOPIC: SCIENCE  
AND BUSINESS IN PHARMACEUTICALS  
FOR THE BENEFIT OF PATIENTS**



# es at a glance

## INVOLVED REGULATORY AGENCIES:

EUROPEAN MEDICINES AGENCY

MEDICINES & HEALTHCARE PRODUCTS REGULATORY AGENCY (ROMANIA)

AGENCE FRANÇAISE DE SÉCURITÉ SANITAIRE DES PRODUITS DE SANTÉ (FRANCE)

AGENCE FÉDÉRALE DES MÉDICAMENTS ET DES PRODUITS DE SANTÉ (BELGIUM)

BUNDESINSTITUT FÜR ARZNEIMITTEL UND MEDIZINPRODUKTE (GERMANY)

POLISH NATIONAL MEDICINES INSTITUTE (POLAND)

NATIONAL INSTITUTE OF PHARMACY (HUNGARY)

FOOD AND DRUG ADMINISTRATION (USA)

ISTITUTO SUPERIORE DI SANITÀ (ITALY)

AGENZIA ITALIANA DEL FARMACO (ITALY)

APIES  
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TOPIC: INNOVATION AND  
BIOTECHNOLOGIES

TOPIC: EVIDENCES FOR  
RATIONAL THERAPIES

ROME  
2011

BARI  
2013

WARSAW  
2012

E, REGULATION  
PHARMACOTHERAPIES  
FIT OF PATIENTS



6<sup>th</sup> Foresight Training Course





# “BIOTECH AND INNOVATIVE SCIENCE TO MEET PATIENT NEEDS”

## HOW TO REACH BARI

### By plane

There are direct flights from Bari to: BARCELONA, BOLOGNA, BRUSSELS CHARLEROI, BUCAREST, BUDAPEST, CAGLIARI, CATANIA, CLUJ, DUSSELDORF WEEZE FRANKFURT HAHN, GENOVA, IBIZA, KARLSRUHE-BADEN, LONDON GATWICK, LONDON STANSTED, LUXEMBOURG, MAASTRICHT, MALTA, MIKONOS, MILANO LINATE, MILANO MALPENSA, MILANO-BERGAMO, MUNICH, MOSCHOW DME, MOSTAR, PALERMO, MAIORCA, PARIS PARIS BEAUVAIS, PISA, PRAGUE, RIGA, ROMA CIAMPINO, ROMA FIUMICINO, SANTORINI, SEVILLE, STUTTGART, TIRANA, TORINO, TRIESTE, VALENCIA, WARSAW CHOPIN, WARSAW MODLIN, VENEZIA, VENEZIA-TREVISO, VERONA, ZURIGO.

## COURSE LOCATION

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