



2/4 September 2009

Pavia • Italy

II Foresight

Training Course

Advanced Therapies and Orphan Drugs

Innovative therapies
in clinical practice:
from birth to risk
management plans
and registries as tools
for safe and timely
available innovation

"Scarpa" Room

University of Pavia

University Building

Strada Nuova, 65 • Pavia

Dear Colleague,

we are pleased to invite you to the second "Foresight Training Course - Advanced Therapies and Orphan Drugs" to be held in Pavia next 2-4 September 2009.

The Course is organized by the Gianni Benzi Pharmacological Research Foundation, in collaboration with SIAR, the Italian Society of Regulatory Activities, CVBF, Consortium for Biological and Pharmacological Evaluations, TEDDY, the Task-force in Europe for Drug Development for the Young, the Master in Regulatory Sciences "GIANNI BENZI" (University of Pavia), EUROPABIO, The European Association for Bioindustries and MEGRA, Middle European Association for Regulatory Affairs.

The second Foresight Training Course, in line with other Gianni Benzi Foundation's initiatives promoting Pharmacological Research and Innovation, will deal with the newest fields developed in the last years and particularly Advanced Therapies, Orphan Drugs and Paediatric Medicines.

EACCME credits will be available.

The Course will provide participants with the opportunity to personally meet some of the members of the EMEA Committee for Advanced Therapies (CAT), Committee for Orphan Medicinal Products (COMP) and Paediatric Committee (PDCO), pose their questions and present their experiences.

Moreover, it will be possible to participate in dinners with the speakers, an occasion for all participants to continue the discussion and analyse in more detail the issues tackled in the programme.

For organisational reasons, we kindly ask your cooperation in confirming your participation by filling in and returning by fax (+39 0382 536544) the attached registration form by Thursday 20th August 2009.

With our best regards,

Adriana Ceci
Gianni Benzi Foundation President

Enrico Bosone
SIAR representative in the Management Board

Gianni Benzi Pharmacological Research Foundation

The Gianni Benzi Pharmacological Research Foundation is a registered "not for profit" Scientific Research Organisation, founded in 2007. Its mission is to contribute to the advancement of pharmacological research in those fields where the scientific heritage of Prof Gianmartino Benzi is more prominent. The main scientific interest areas of Prof Benzi were neurobiology and neuropathology of degenerative disorders as well as the effects and the risks of drugs used as doping. Noticeable these scientific competences have been usefully merged with his relevant regulatory experience at the EMEA aimed at setting up a competitive and advanced Pharmaceutical System in Europe.

Gianni Benzi Foundation objectives and fields of activities

To achieve its mission, the Foundation will undertake a range of projects and work.

In particular, initiatives will support the following scientific areas: ***Regulatory aspects at European level, Innovative and orphan drugs development, Safety of drugs, Drugs abuse and misuse including Doping***

Activities:

- Research programs through operating grants directly allocated to research project
- Educational programs, including primary and secondary level school
- Public surveys and campaigns on aspects of social interest
- Scientific and popular publications
- Grants for students and young researchers
- Scientific events and Conferences





2 / 4 S e p t e m b e r 2 0 0 9 • P a v i a • I t a l y

II Foresight Training Course

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Innovative therapies
in clinical practice:
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management plans
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Course Directors

Enrico Bosone

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

Mariana Catapano

(Head of Regulatory Affairs, CVBF)

Scientific Committee

Vittorio Silano *(President of Scientific Committee, Gianni Benzi Foundation)*

Paola Baiardi *(Director, CVBF)*

Walter Bianchi *(President, SIAR)*

Diana Conte Camerino *(Professor of Pharmacology, University of Bari)*

Liliana Di Ciano *(QR, Advanced Therapies Center, IRCCS Multimedica)*

Beatrice Jaha *(Member of MEGRA, MB and RA Manager, Celgene Germany)*

Jean-Pierre Osselaere *(Managing Director, EPMC Pharma)*

Marcello Tonini *(Director, Master "Regulatory Sciences - Gianni Benzi")*

Organising Secretariat

Consortium for Biological and Pharmacological Evaluations

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E-mail: secretary@benzifoundation.org

Gianni Benzi Foundation thanks the following Companies which have contributed to the implementation of the Course:

Abiogen Pharma, Amgen Dompé, Boehringer Ingelheim, Bristol - Myers Squibb, Celgene,

Chiesi Farmaceutici, Dompé Farmaceutici, Gentium, Gilead, Kedrion, Novartis Farma,

Pfizer, Roche, Rottapharm, Sanofi - Aventis, Scharper, Schering - Plough, Sigma - Tau, UCB.

B a c k g r o u n d

Scientific progress provides new opportunities to respond to unmet medical needs. Selective incentives provided by the European Regulations, drive the research towards fields with most therapeutic needs and towards rare and neglected diseases, which without bright social policies would remain at the border of the process, devoid of the advantages coming from technology improvements. If the number of patients is no longer the only or the main parameter which drives research strategies, therapies are going to become personalized thanks to the growing knowledge in the fields of genetic and molecular chemistry and biochemistry. The second Foresight Training Course, in line with other Gianni Benzi Foundation's initiatives promoting Pharmacological Research and Innovation, will deal with the most innovative fields developed in the last years and particularly Advanced Therapies, Orphan Drugs and Paediatric Medicines.



T o p i c s

The European Regulation on advanced therapies entered into force in January 2009. It includes gene therapy, somatic cell therapy and engineered tissues. These products must follow the European centralized procedure and a new Committee has been set up to evaluate them in collaboration with the CHMP: the Committee on Advanced Therapies (CAT). The new procedure will be analyzed

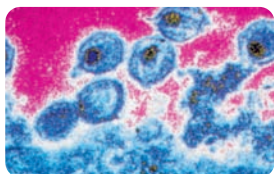
during the Course with a discussion on the available examples and emerging problems. More than 50 orphan drugs have been approved for use in Europe and today they constitute a solid reality. All the relevant procedures will be examined: from the designation, through the "protocol assistance" and evaluation during the centralized procedure, to the availability in the European Countries,

an unsolved problem with some interesting proposals under discussion. Starting from the innovations introduced by the recently approved European Paediatric Regulation, a special session will be devoted to innovation in the Paediatric Medicines field. The drug "post-marketing" phase has now an

increased meaning in terms of better knowledge of the safety profile, including rare adverse events which cannot be detected during the "development phase", the appropriate use and the better understanding of the real drug "added-value". Representatives of the EMEA, the

Italian Medicines Agency (AIFA) and other National

Agencies will participate in the Gianni Benzi Foundation second Foresight Training Course, together with many other very experienced speakers from the Academy, Public Bodies and Pharmaceutical Industry.



9.00 Course Presentation *Walter Bianchi, Adriana Ceci, Marcello Tonini*

9.30 Welcome to the Course *Representatives of the Authorities*

Opening Session 1

Chairs: *Adriana Ceci, Christian Schneider*

10.00 Public and Private efforts to develop ODs and Advanced Therapies Medicinal Products (ATMPs)

EMA initiatives for innovation *Agnés Saint-Raymond*

Promoting innovation at National Level *Guido Rasi*

11.00 Coffee break

Opening Session 2

Chairs: *Vito Campese, Claudio Cavazza*

11.30 Public and Private efforts to develop Orphan Drugs (ODs) and Advanced Therapies Medicinal Products (ATMPs)

The role of the Pharmaceutical Industries *Sergio Dompé*

The EU Innovative Medicines Initiative *Giovina Ruberti*

Telethon experience and role *Carla Paganin*

The FDA experience *Linda Ulrich*

12.45 Speakers and Experts answer your questions (with PRESS participation) *All speakers*

13.30 Lunch

Advanced Therapies: Session 1

Chair: *Enrico Bosone, Jordi Llinars Garcia*

15.00 Rules and tests to develop ATMPs in the context of the EU Regulation

Critical points and perspectives *Christian Schneider*

Rules and experience *Liliana Di Ciano, Marco Dieci, Annarita Meneguz*

16.00 Speakers and Experts answer your questions *All speakers*

16.30 Coffee break

Advanced Therapies: Session 2

Chair: *Franco Locatelli, Jean-Pierre Osselaere*

16.45 Scientific aspects of advanced therapies, classification of ATMPs, consistency and reliability of the results

Gene and Cell Therapy *Giovanni Migliaccio*

Mesenchymal stem cell as therapeutics *Michael Siani-Rose*

Gene therapy for Thalassemia: a US - Italy project *Aurelio Maggio*

17.45 Speakers and Experts answer your questions *All speakers*

18.15 Remarks and conclusions *Christian Schneider*



Orphan Drugs: Session 1

Chair: *Giuseppe Nisticò, Vittorio Silano*

9.00 Scientific Advice for ODs

Critical points and perspectives *Jordi Llinares Garcia*

Experiences *Katrin Rupalla, Jean-Pierre Osselaere*

10.00 Planning and submitting clinical studies

Clinical trials design and management *Paola Baiardi*

Modelling and population PK in orphan/paediatric diseases *Oscar Della Pasqua*

ODs for primary immunodeficiencies *Alessandro Aiuti*

ODs in metabolic diseases *Carlos Camozzi*

ODs in haematology *Paola Zanon*

Orphan Drugs: Session 2

Chair: *Enrico Bosone, Diana Conte Camerino*

12.00 ODs assessment in the centralized procedure: conditional and exceptional approvals and FollowUp Measures

Critical points and perspectives *Giuseppe Nisticò*

Rules and experiences *Chris Walker*

12.30 Speakers and Experts answer your questions *All speakers*

12.45 Remarks and conclusions *Giuseppe Nisticò, Jordi Llinares Garcia*

13.00 Lunch

Orphan Drugs: Session 3

Chair: *Andrea Rappagliosi, Domenica Taruscio*

15.00 Designation: plausibility, protocol assistance, clinical benefit, similarity, re-assessment

The COMP perspectives *Kerstin Westermark*

Rules and experiences *Jean-Pierre Osselaere, Chris Walker*

Consideration around development of ODs for children *Catarina Edfjaell*

16.30 Coffee break

Orphan Drugs: Session 4

Chair: *Stefano Govoni, Roberto Gradnik*

16.45 Clinical practice and availability

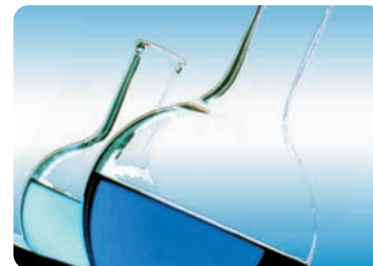
The experience with the post - marketing registries *Domenica Taruscio*

ODs prescription data in Europe *Miriam Sturkenboom*

Post-marketing Registries and ODs safety *Carlo Tomino*

17.30 Speakers and Experts answer your questions *All speakers*

18.00 Remarks and conclusions *Domenica Taruscio, Kerstin Westermark*



Focus on Paediatrics

Medical needs in Paediatrics including ODs and ATMPs: hopes and realities

Chair: *Paolo Rossi, Agnès Saint-Raymond*

- 9.00** Critical points and perspectives on Paediatric Investigation Plans (PIPs) for developing Innovative Medicines *Daniel Brasseur*
- 9.30** Rules and experiences
 - Off-patent drugs and PUMA *Vincent Grek*
 - Paediatric Orphan Drugs *Mariana Catapano*
 - Medicines development in neonates: the TINN experience *Evelyne Jacqz-Aigrain*
- 10.30** Speakers and Experts answer your questions *All speakers*
- 10.45** *Coffee break*

Medical needs in Paediatrics including waivers and class waivers

Chair: *Fergal Donnelly, Marcello Tonini*

- 11.00** Needs and PIPs by Therapeutic Classes *Paolo Rossi*
 - **Rheumatology** *Nicola Ruperto*
 - **Oncology** *Paolo Paolucci*
 - **Vaccines** *Eddie Reilly*
 - **Paediatric intensive care** *Marek Migdal*
- 12.15** Speakers and Experts answer your questions *All speakers*
- 12.45** Remarks and conclusions *Daniel Brasseur*
- 13.00** *Lunch*

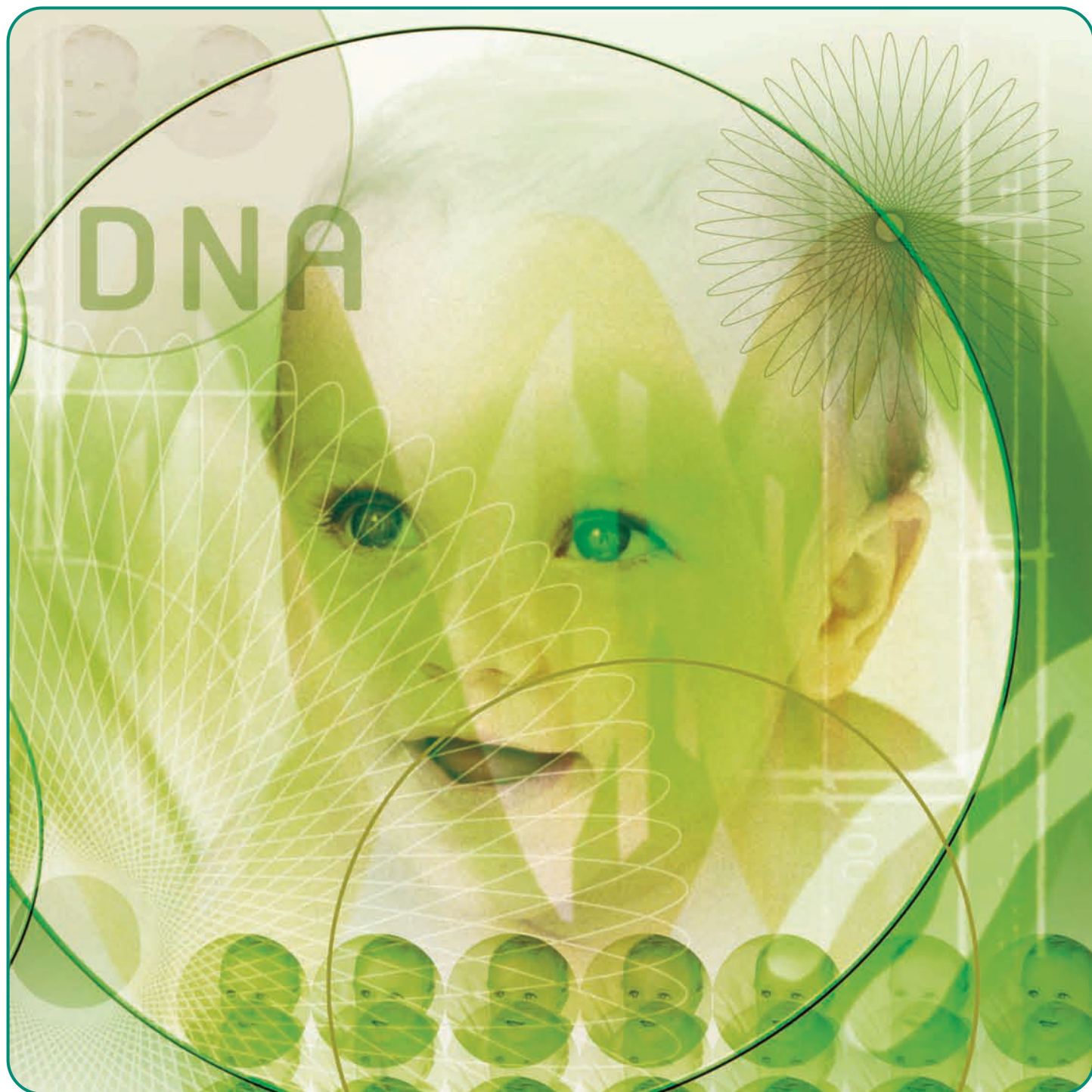


Paediatric Medicines facing Innovation: are there differences worldwide?

Chair: *Adriana Ceci, Daniel Brasseur*

- 14.00** Introductory remarks *Fergal Donnelly*
- 14.30** Critical points for paediatric research
 - Networks sustainability
 - Ethical approaches
 - Recruitment policies
 - End-points validation
 - Research Priority Agenda
 - Costs and return on investments
 - The need for coordinated educational initiatives

Carlo Giaquinto, Steven Hirschfeld, Hidefumi Nakamura, Linda Ulrich, John van den Anker
- 16.00** Speakers and Experts answer your questions (with PRESS participation) *Experts from Industry, TEDDY, EMEA/PDCO*
- 17.00** Remarks and conclusions *Carlo Giaquinto, Fergal Donnelly, Agnès Saint-Raymond*
- 17.30** Course Conclusions



Registration Form

Please return the form to GIANNI BENZI FOUNDATION
Tel. +39 0382 25075 • Fax +39 0382 536544 • mail:secretary@benzifoundation.org

Name _____ Surname _____
Date and place of birth _____ Taxpayer number _____
Address _____
Post code _____ Town _____ Country _____
Tel. _____ Fax _____ E-mail _____
Organisation _____ Job title _____
Address of Organisation _____
Post code _____ Town _____ Country _____
Tel. _____ Fax _____ E-mail _____
VAT number _____ Taxpayer number _____

Registration Fees (VAT excluded)	Full Corse	Full Corse (Registration by 31/7/09)	Full Corse + Dinners	Full Corse + Dinners (Registration by 31/7/09)
Standard	Euro 2300,00	Euro 2150,00	Euro 2550,00	Euro 2400,00
Members of Regulatory Affairs Societies SIAR and MEGRA	Euro 2150,00		Euro 2400,00	Euro 2150,00
Graduate Students*	Euro 1800,00		Euro 2050,00	Euro 1800,00

*Students must DEMONSTRATE enrolment to Masters in related topics

Method of Payment

Bank transfer to: Gianni Benzi Foundation
IBAN code: IT 63 I 01010 41750 100000001097 – 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 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 Pavia

Milano Linate and ***Milano Malpensa*** are the nearest airports.

Arriving at Milano Linate

Milano Linate airport is connected to Pavia through a bus service operated by Migliavacca.

www.migliavaccabus.it

Arriving at Milano Malpensa

There is a bus service that connects the airport to the Milano Centrale Railway Station.

Buses leave from Malpensa to Milano Centrale every 20 min.

Alternatively, it is possible to reach Milano with the Malpensa Express Train to Milano Cadorna Railway Station where the Underground system can be used to get to the Milano Centrale Railway Station.

Trains from Malpensa airport leave every 30 min.

By train

Pavia is served by frequent trains from Milano Centrale Railway Station.

www.trenitalia.com

Hotels In Pavia

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viale Vittorio Emanuele II, 41 • Tel: +39 0382 303401

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via Lombroso 21/23 • Tel: +39 0382 526312

S p e a k e r s L i s t

Alessandro Aiuti

Associated Professor of Paediatrics, University of Tor Vergata - Rome, and Head of Unit, Istituto San Raffaele Telethon per la Terapia Genica (HSR - TIGET), Italy

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Executive Board Member, Task-force in Europe For Drug Development for the Young (TEDDY) Network of Excellence, and Director, Consortium for Biological and Pharmacological Evaluations (CVBF), Italy

Walter Bianchi

President, Italian Society of Regulatory Activities (SIAR), Italy

Enrico Bosone

Director, Regulatory Affairs & Compliance, Celgene Italy and SIAR Member of MB, Italy

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Chair, Paediatric Committee (PDCO), European Medicines Agency (EMA), and Fédérale des Médicaments et des Produits de Santé, Belgium

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Claudio Cavazza

President, Sigma-Tau, Italy

Adriana Ceci

President, "Gianni Benzi" Pharmacological Research Foundation, Italy

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Professor of Pharmacology, University of Bari, Italy

Oscar Della Pasqua

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

Liliana Di Ciano

Qualified Person, Advanced Therapies Center, IRCCS Multimedica, Italy

Marco Dieci

Operations Director, MolMed, Italy

Sergio Dompé

President, Farminindustria, Italy

Fergal Donnelly

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

Catarina Edfjaell

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

Carlo Giaquinto

Co-coordinator, Task-force in Europe For Drug Development for the Young (TEDDY) Network of Excellence, and Director of the Paediatric AIDS and Paediatric Clinical Research Unit, Azienda Ospedaliera Padova, Italy

Stefano Govoni

Professor of Pharmacology, University of Pavia, and Director, Master Marketing Farmaceutico, University of Pavia, Italy

Roberto Gradnik

Executive Vice President, Commercial Europe, Merck Serono and President of the Italian Biotech Industry Association, Assobiotech, Italy

Vincent Grek

CEO and Chief Medical Officer, Only for Children Pharmaceuticals, France

Carlos Camozzi

Medical Director, Orphan Europe, France

Steven Hirschfeld

Associate Director for Clinical Research, Eunice Kennedy Shriver National Institute of Child Health and Human Development, USA

Evelyn Jacqz-Aigrain

Director, Department of Paediatric Pharmacology and Pharmacogenetics "Robert Debré" Hospital, France

Beatrice Jaha

Manager, Regulatory Affairs, Celgene Germany, and Member of MEGRA MB, Germany

Jordi Llinares Garcia

Scientific advice and orphan drugs Sector, European Medicines Agency (EMA), UK

Franco Locatelli

Director, Paediatric Oncology/Haematology Unit, Fondazione IRCCS Policlinico San Matteo, Italy

Aurelio Maggio

Director, Division of Haematology II with Thalassemia, "V. Cervello" Hospital, Italy

Annarita Meneguz

Member of the Safety Working Party (SWP), European Medicines Agency (EMA), and Director of Biochemical Pharmacology Department, Istituto Superiore di Sanità, Italy

Marek Migdal

Member Paediatric Committee (PDCO), European Medicines Agency (EMA), and Deputy Head of PICU, Children's Memorial Health Institute, Poland

Speakers List

Giovanni Migliaccio

Italian Member of the Committee on Advanced Therapies (CAT), European Medicines Agency (EMA), and Head of Research, Istituto Superiore di Sanità, Italy

Hidefumi Nakamura

Director, Division of Clinical Research and Office for Drug Evaluation, National Childrens Medical Center, National Center for Child Health and Development, Japan

Giuseppe Nisticò

Member of the Management Board and the Committee for Medicinal Products for Human Use (CHMP), European Medicines Agency (EMA) and Italian Medicines Agency (AIFA), Italy

Jean-Pierre Osselaere

Managing Director, EPMC Pharma, Belgium

Carla Paganin

Regulatory and Research Program Manager, Comitato TELETHON Fondazione Onlus, Italy

Paolo Paolucci

Professor of Paediatrics and Head Department, University of Modena and Reggio Emilia, Italy

Andrea Rappagliosi

Vice President, European Government Affairs & Head of Brussels Office, GlaxoSmithKline, and member of the Executive Committee, Europabio, Belgium

Guido Rasi

General-Director, Italian Medicines Agency (AIFA), Italy

Eddie Reilly

Head EU & International RA, Vaccines and Diagnostics, Italy

Paolo Rossi

Member of the Paediatric Committee (PDCO), European Medicines Agency (EMA), and Head of the Division of Immunology and Infectious Disease, "Bambino Gesù" Children's Hospital, Italy

Giovina Ruberti

Italian States Representatives Group (SRG), Innovative Medicines Initiative (IMI), European Commission, and Head of Research, Institute of cell biology, National Research Council (CNR), Italy

Nicola Ruperto

PRINTO Senior Scientist, IRCCS G. Gaslini Hospital, Genoa, Italy

Katrin Rupalla

Senior Director, Regulatory Affairs, Celgene International, Switzerland

Agnès Saint-Raymond

Head of Scientific Advice, Paediatrics and Orphan Drugs Sector, European Medicines Agency (EMA), UK

Christian Schneider

Chairperson, Committee on Advanced Therapies (CAT), European Medicines Agency (EMA), and Acting Head, Division EU Co-operation/Microbiology, Paul-Ehrlich-Institut (PEI) Federal Agency for Sera and Vaccines, Germany

Michael Siani-Rose

President, Theragen, USA

Vittorio Silano

President Scientific Committee, "Gianni Benzi" Pharmacological Research Foundation and President of the EFSA Scientific Commission, Italy

Miriam Sturkenboom

Professor of Pharmaco-epidemiology, Erasmus University Medical Center, The Netherlands

Domenica Taruscio

Director, National Center for Rare Diseases, Istituto Superiore di Sanità, Italy

Carlo Tomino

Director, National Monitoring Centre on Clinical Research with Medicines (OsSC), Italian Medicines Agency (AIFA), Italy

Marcello Tonini

Director, Master in Regulatory Sciences "GIANNI BENZI" and Head of Department of Pharmacology, University of Pavia, Italy

Linda Ulrich

Medical Officer, Office of Orphan Products Development, Food and Drug Administration (FDA), USA

John van den Anker

Director, Pediatric Clinical Pharmacology, Children's National Medical Center, Washington, USA

Chris Walker

Senior Manager Regulatory Affairs, Amgen, USA

Kerstin Westermark

Chairperson, Committee on Orphan Medicinal Products (COMP), European Medicines Agency (EMA), and Adjunct Professor of Medicine, Department of Medical Sciences, Uppsala University, Sweden

Paola Zanon

Medical Director, Celgene, Italy

organised by



in collaboration with



*Master in Regulatory Sciences
"Gianni Benzi" University of Pavia*



with the support of



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