



Gianni Benzi Pharmacological Research Foundation

VII Foresight Training Course

In the context of

NEX-T-WORK

European Projects Forum

Background

The Gianni Benzi Pharmacological Research Foundation (FGB) is a registered not-for-profit scientific research organisation aimed at promoting research in innovative biological, medical and other products for human use. Besides research, the activities of the Foundation are addressed to high level educational initiatives that are mainly devoted at consolidating the existing knowledge and taking into account the current European strategies concerning Regulatory Sciences.

The Gianni Benzi Foundation yearly organises the '**Foresight Training Courses**' (FTCs), that are short intensive courses devoted to create a critical mass of expertise in the different areas of Regulatory Sciences (medical devices, foods, medicinal products), with particular reference to the innovative advancement of the European Pharmaceutical and Biological products sectors. Six Foresight Training Courses have been performed until today with participants from 21 EU/non EU Countries.

The experience of the Foresight Training Courses is mainly aimed at sharing the existing knowledge in the area and fostering the networking of experts from Universities, Industries, and national and EU Regulatory Agencies, all taking an active role in building the new scenario of Regulatory Sciences.

The FTCs also gain their strength from the involvement of experts, decision makers and all interested stakeholders (Regulators, Researchers, Health Professionals, Private and Public Companies, Patient Associations) coming from outside Europe, making them an interesting occasion of debate and a collaborative effort within the existing international networks.

Foresight Training Course 2014

The VII° Foresight Training Course will be held in Castellaneta Marina (Italy), October 2-4, 2014, in the context of the European Projects Forum "**Nex-t-work**".

Nex-t-work represents the right scenario to organise the seventh edition of the Foresight Training Course and the ideal context to share its objectives and its mission with all the stakeholders involved in the evolution of Regulatory Sciences and Human Research.

Nex-t-work is aimed at networking and developing international relations with the various players of the EU grant-funded projects in order to share best practises and stimulate the intersectoral collaboration between academic and educational Institutions, Universities, Companies, Public Authorities, Innovation Centres, Scientific Associations, Regulatory Experts, Research Networks, Investors, Students and Patients.

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Furthermore, the inclusion of the VII Foresight Training Course in the wider scenario of Nex-t-work, in which workshops and training initiatives are scheduled, will offer our guests the possibility to participate in different sessions of their interest, complementary and open to different targets.

Overall, the Gianni Benzi Foundation will participate in organising the following initiatives:

- 1- Project-best practises sessions: two EC-funded projects in which the Foundation is involved (DEEP, DEferiprone Evaluation in Paediatrics and InNerMeD-I-Network) will be presented;
- 2- Training sessions: 'Health, Food Safety and related Policies for the Mediterranean Area (HEFSA)' Master and the 'International Master in Regulatory Sciences' (IMRS) will be officially launched (in collaboration with Ondokuz Mayıs University and CMU-Community of Mediterranean Universities);
- 3- International Conference: Mediterranean, European policies and the new framework 2020 (organised by Nex-t-work);
- 4- Focus session: Progresses and opportunities in conducting Paediatric Trials (organised by TEDDY-European Network of Excellence for Paediatric Clinical Research);
- 5- Foresight "Health and Biotechnology": novelties in regulatory field will be discussed, such as the European Reference Networks (ERNs), the proposal for a Good Genomics Practices (GGP) guidelines, the Scientific Advice applications.

You are kindly invited to participate in these initiatives that will be held in Castellaneta Marina (Taranto -Italy), October 2-4 2014.

The President

Prof. Adriana Ceci

Gianni Benzi Pharmacological Research Foundation

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INHERITED NEUROMETABOLIC DISEASES INFORMATION NETWORK - InNerMeD-I-Network

Thursday, October 2nd 2014

10.00-11.00

InNerMeD-I-Network is a European project funded by the Executive Agency for Health & Consumers (DG SANCO) under the Second Programme to be the first European Information Network on Inherited NeuroMetabolic Diseases (iNMDs). It started on the 1st of April 2013.

InNerMeD-I-network wants to create a multimedial network of information targeted on diagnosis and treatment of iNMDs and based on the collection and exchange of validated information among scientific communities, health professionals, patients associations and all interested stakeholders.

iNMDs are a group of rare genetic metabolic diseases that impact on the brain causing mental retardation and progressive neurodegeneration which, if not promptly treated, could end in early death.

The project aims to increase current knowledge on iNMDs and speed up the timely and precise identification of patients who may benefit of the available treatments (experimental and marketed). The network will also favour biomedical research, straightening research capacities and fostering innovative therapeutic tools derived from the recent scientific advancements.

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DEFERIPRONE EVALUATION IN PAEDIATRICS - DEEP

Thursday, October 2nd 2014

11.00 – 12.00

The main goal of the DEEP project is the development of a new formulation of deferiprone for the treatment of iron overload in paediatric patients affected by congenital, transfusion-dependent anaemias such as β -thalassaemia major, also known as “Mediterranean anaemia”, and sickle cell disease (SCD).

The project addresses one of the specific paediatric problems highlighted in the priority list of PDCO (the Paediatric Committee at the European Medicines Agency) in 2009 and has been funded by the European Commission (FP7).

In particular, the intention to fulfil the ambitious objective of the project is deeply pursued throughout the conduction of two clinical trials and one observational study, in accordance with a Paediatric Investigation Plan (PIP) approved by the EMA Paediatric Committee. At the end of the project an application to obtain a Paediatric Use Marketing Authorisation (PUMA) for the new deferiprone paediatric formulation will be submitted.

16 Partners from 6 EU countries and 4 non EU are involved and the Policlinic of Bari is performing a key-role in terms of management and clinical activities, in particular acting as the Coordinating Centre of the DEEP-1 Clinical Trial and hosting the Drug Management Service for all the Italian clinical centres involved.

The first clinical trial, the DEEP-1 PK-study, was concluded on February 2014 and allowed to define the correct dosage of deferiprone to be administered to patients aged from 1 month to 6 years. This preliminary result has been essential for the treatment of the young patients to be enrolled in the second and most important clinical trial envisaged by the DEEP Project set-up: DEEP-2 Efficacy/Safety Study.

DEEP-2 is a multicentre, randomised, open label, non-inferiority active-controlled clinical trial to evaluate the efficacy and safety of deferiprone, in comparison with the standard of care, deferasirox. The recruitment plan intends to involve 344 paediatric patients in 18 clinical centres located in both EU and non-EU countries: Albania, Cyprus, Greece, Egypt, Italy, Tunisia and UK.

Five different Italian sites have already started the enrolment so far, and the very next ones on the brink to enrol are the Egyptian and Albanian experimental sites. DEEP-2 represents the second clinical trial ever performed in Albania.

DEEP-3 Long term Safety Study aims at evaluating the safety of deferiprone in children with beta-thalassaemia major, through the collection and observation of chelation-therapy-related data, both retro- and prospectively, from the beginning of deferiprone assumption. 16 centres located in Albania, Cyprus, Egypt, Greece, Italy and Tunisia are actively participating; the observation has already been completed for 37 subjects and will continue up to reach the planned sample of 400 screened subjects.

Special attention has been dedicated throughout the project to the development of age-tailored informative tools specifically targeted on the paediatric patients involved in the studies.

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OFFICIAL PRESENTATION OF THE INTERNATIONAL MASTERS ON

Health, Food Safety and related Policies for the Mediterranean Area (HEFSA)

International Master in Regulatory Sciences (IMRS)

*Promoted by Community of Mediterranean Universities, Ondokuz Mayıs University (Turkey), Gianni Benzi
Pharmacological Research Foundation*

Thursday, October 2nd 2014

16.30 – 18.30

Nex-t-work will be the occasion to officially launch the two international Masters: the International Master in Regulatory Sciences and the Health, Food Safety and related Policies for the Mediterranean Area Master, that the Gianni Benzi Foundation is organising in collaboration with European and non European Universities and with the Community of Mediterranean Universities (CMU), a consortium aimed at promoting scientific cooperation among Mediterranean Universities.

The Masters that will be presented are:

HEFSA. "Health, food safety and related Policies in the Mediterranean Area" Master aims to train professionals in the agricultural and medical-biological sectors, able to act at any level, including Public Health Institutions, to promote correct agricultural, social-health, food and nutrition strategies and policies even providing opinion on how to comply with current regulations.

IMRS. The International Master in Regulatory Sciences aims to create and update highly experienced professionals in the evolving field of Regulatory Science, to provide them full and update knowledge at European/non-European level. It represents an innovative training tool both for the contents and the methodology. It is focused on Regulatory Sciences, a discipline combining regulatory issues related to the development of a biomedical product with scientific research, necessary to ensure the safety and efficacy of these products.

Agenda

16.30 Session 1: Internationalisation of higher education

Chairs: *F. Losurdo - H. Mennan*

- The promotion of the scientific cooperation among Mediterranean Universities: The Community of Mediterranean Universities vision (*G. Saglamer*)
- The role of high level training in Health and Food Safety (*C. Di Benedetta*)
- The role of high level training in Regulatory Sciences (*A. Ceci*)

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17.30 Session 2: International Masters presentation

Chairs: *V. Silano- L. Trabace*

- Presentation of the international Master on Health, Food Safety and related Policies for the Mediterranean Area” (*L. Ricciardi*)
- Presentation of the International Master in Regulatory Sciences (*M. Lupo*)
- The Industry’s expectations and needs (*A. Natz/D. Criscuolo*)
- The Academy’s expectations and needs (*A. Altavilla*)
- How to deal with the National and International Agencies’ requirements (*V. Salvatore, Turkish representative, tbd*)

Discussion

Chairs and Speakers:

Annagrazia Altavilla (Espace Éthique Méditerranéen; Aix-Marseille University - AMU, France)

Adriana Ceci (President of the Gianni Benzi Pharmacological Research Foundation – FGB, Scientific Coordinator of the International Master on Regulatory Science, Italy)

Domenico Criscuolo (President of Genovax, Italy)

Carlo Di Benedetta (Coordinator of the Head Office of Community of Mediterranean Universities – CMU, Italy)

Francesco Losurdo (General Secretary of Community of Mediterranean Universities - CMU, Italy)

Mariangela Lupo (Gianni Benzi Pharmacological Research Foundation – FGB, Italy)

Hüsrev Mennan (Pro-Rector, International Relations Office, University of Ondokuz Mayıs – OMU, Turkey)

Alexander Natz (Secretary General of the European Confederation of Pharmaceutical Entrepreneurs – EUCOPE, Belgium)

Luigi Ricciardi (University of Bari - UNIBA, Italy)

Gulsun Saglamer (President of the Community of Mediterranean Universities- CMU, Turkey)

Vincenzo Salvatore (University of Insubria, Professor of International Law, Italy)

Vittorio Silano (President of the Scientific Committee of the Gianni Benzi Pharmacological Research Foundation – FGB, Italy)

Luigia Trabace (University of Foggia – UNIFG, Italy)



International Conference
“Mediterranean, European policies and the new framework 2020”

Friday, October 3rd 2014

11.00 - 13.00

The **Europe 2020 strategy** aims to boost the EU economy over the next decade in order to become smart, sustainable and supportive. These three priorities are mutually reinforcing and aim to help the EU and the Member States to achieve high levels of employment, productivity and a more social cohesion.

In particular, the European Union has set five ambitious objectives, to be reached by 2020, in the field of **employment, innovation, education, social inclusion and climate/energy**.

The international conference on “Mediterranean, European policies and the new framework 2020” is aimed at discussing the main challenges that the Italian Presidency is already facing as well as the Mediterranean as a place of cooperation and a new model of economic and social development to which all are called to participate.

It will focus on the following main topics:

- the Europe 2020 Strategy, to better understand how to effectively achieve the primacy of small and medium industries, that have always been the backbone of the European economy as well as the policies to improve industrial competitiveness;
- actions in favour of unemployment, with particular reference to the youth, through mobility, that has to be fostered through social dialogue, creation of new jobs, structural reform of labour market and the investment in human capital;
- the creation of new jobs and a new entrepreneurs class based on new technologies and the developments of the digital communication;
- new employment opportunities in the agricultural sector through investments in agro-industrial, tourism, catering and initiatives to protect the countryside;
- how to promote tourism and culture, through projects and policies aimed to enhance the cultural and territorial heritage;
- policies for research and innovation that are the pillars to develop a sustainable growth and are today more and more strictly connected with improvement of health, services for elderly people and biotechnologies.

The international conference will benefit from speakers coming from the European Parliament, the Italian Government, the National Research Council (CNR), the Presidency of the Apulia, Campania and Basilicata Regions, Industry and Academy.



**TEDDY: A EUROPEAN NETWORK OF EXCELLENCE
FOR PAEDIATRIC CLINICAL RESEARCH**

Organised by CVBF-TEDDY

in the context of GRiP Project – Global Research in Paediatrics – FP7 n° 261060

Friday, October 3rd 2014

14.30 – 17.30

TEDDY is a European Paediatric Network funded under the EC-FP6 Program in 2005 as an Excellence Research Network aimed at developing paediatric medicines research and innovative studies. After expiring the funded period, TEDDY has consolidated its activities in the field through a research Consortium Agreement among 41 partners from 14 countries, coordinated by Consorzio per Valutazioni Biologiche e Farmacologiche (CVBF), that is also involved in the GRiP Project as CVBF-TEDDY.

This meeting is proposed as the first General Assembly after the agreement has been signed and will be the occasion for the nomination of the scientific bodies of the network. It will include an update on the relevant innovative initiatives of interest for the paediatric research in Europe and a posters session directly promoted by the researchers participating in the network.

Agenda

Chairs: *D. Bonifazi – I. Grosch- Wörner*

- TEDDY experience in developing paediatric research in Europe and Horizon 2020 (*A. Ceci*)
- From DEEP to CloSed: the pathway of the University Hospital Erlangen to become in two years Coordinator of a FP7 Project along with the experience of Teddy Network (*A. Neubert, tbc*)
- Paediatric Clinical Trials in Albania: in the absence of a regulation, Big Pharma give way to research-driven EU funded projects: the safety-efficacy paediatric clinical trial in thalassaemia (*M. Kreka*)
- A Joint Degree in Paediatric Pharmacology and Clinical trials in children: an initiative of GRiP - the Global Research in Paediatrics Network of Excellence (*P. Rossi*)
- Promoting a cost-efficient strategy for people with Autism Spectrum Disorder (ASD) across Europe – PASDE: a new proposal under Horizon 2020 developed into the TEDDY Network (*A. Tarpan*)
- GRiP and EnprEMA, the European Networks for Paediatric Research (*C. Giaquinto*)
- Innovative studies designs to favour paediatric research (*P. Baiardi*)
- TEDDY infrastructure, database and SOPs for paediatric clinical trials (*C. Manfredi*)

16.30 Posters (or short communications) session: TEDDY researchers sharing proposals and results (*L. Ruggieri*)



Chairs and Speakers:

Paola Baiardi (GRiP Project WP Leader Work Package 4 “New Methods for Clinical Studies in Paediatrics)

Donato Bonifazi (*Managing Director of Consorzio per Valutazioni Biologiche e Farmacologiche – Bari, Italy*)

Adriana Ceci (*Former Coordinator of the Network of Excellence TEDDY, President of the Gianni Benzi Pharmacological Research Foundation – Valenzano, Italy*)

Carlo Giaquinto (*GRiP Coordinator, President of Fondazione Penta, - Padova, Italy*)

Ilse Grosch- Wörner (Charite Universitätsmedizin, Berlin, Germany)

Manika Kreka (*DEEP-2 Principal Investigator, Mother Teresa University Hospital – Tirana, Albania*)

Cristina Manfredi (*Consorzio per Valutazioni Biologiche e Farmacologiche – Pavia, Italy*)

Antje Neubert (*CloSed Project Scientific Coordinator, University Hospital Erlangen - Germany*)

Paolo Rossi (*Head of the Paediatric Department at the University Hospital Bambino Gesù – Rome, Italy*)

Lucia Ruggieri (*Italian Group for Pharmacoeconomic Studies – Valenzano, Italy*)

Adelaide Katerine Tarpan (Romanian Angel Appeal Foundation – Bucarest, Romania)



FORESIGHT

“HEALTH AND BIOTECHNOLOGY”

Saturday, October 4th 2014

09.00 – 13.00

High-level educational activities in the field of health and biotechnology are strongly requested to grant the ability to create international, shared, successful research experiences to the professionals. These training activities should encompass scientific and regulatory aspects concerning:

- new management aspects of the public and research Institutions with particular reference to the development of the European Reference Networks (ERNs);
- new ERNs development on Rare Diseases and new proposals;
- ICT role in networking and research and the development of new networks;
- collaboration among researchers, managers of small and big companies in the field of biotechnology;
- scientific paths based on the evidences for the preparation of guidelines on Good Genomic Practises (GGP).

The course will provide 4,7 Italian ECM credits for Healthcare Professionals.

Agenda

09.30 Session 1: European Reference Networks (ERNs): ‘A new vision for patients’ health’

Chairs: *R. Barbon Galluppi, A. Ceci*

- ERN: Procedures, competences, skills in the European vision (*A. Ghirardini*)
- The European Reference Networks for Rare Diseases (*D. Taruscio*)
- Excellence to be translated in a new ERN (*M. Migdal/A. Maggio*)
- ICT role in Networking and research (*G. Visaggio*)

11.00 Session 2 : Role and relevance of Biotechnology in a growing society

Chairs: *E. Milella, M. Svelto*

- The key role of high technology districts and clusters in the national and international framework. The H-BIO experience (*M. Svelto*)
- Successful Biotech initiatives: Masmec (*A. M. Vinci*), Dompè (*M. Allegretti*)
- Biomarkers clinical use: from laboratory to the product (*M. Papale*)
- Concept paper on good genomics biomarker practices: how to prepare a guideline for researchers, industry and patients? (*E. Bosone*)

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Chairs and speakers

Marcello Allegretti (*Chief Scientific Officer at Dompè SpA, Italy*)

Enrico Bosone (*Director of Regulatory Affairs and Compliance at Celgene, Board of Directors Representative at SIAR, Italy*)

Adriana Ceci (*President of the Gianni Benzi Pharmacological Research Foundation – FGB, Italy*)

Renza Barbon Galluppi (*President of Uniamo Federazione Italiana Malattie Rare Onlus – UNIAMO FIRM, Italy*)

Loreto Gesualdo (*Director of the Nephrology Residency Program, School of Medicine, University of Bari, Italy*)

Alessandro Ghirardini (*Office III Director – DG della programmazione sanitaria, Ministry of Health, Italy*)

Aurelio Maggio (*Director of U.O.C Ematologia e malattie rare del sangue e degli organi ematopoietici, Ospedali Riuniti Villa Sofia – Cervello – AORC, Italy*)

Marek Migdal (*Deputy Head, PICU, PDCO EMA member, Children's Memorial Health Institute, Poland*)

Eva Milella (*President of the Regional Agency for Technology and Innovation – ARTI, Italy*)

Massimo Papale (*Researcher at University of Foggia; scientific consultant at Bio2xygen srl*)

Maria Svelto (*President of the High Technology District H-Bio Puglia – H-BIO Puglia, Italy*)

Domenica Taruscio (*Director of the Italian National Centre for Rare Diseases, Italian National Institute of Health – ISS, Italy*)

Angelo Michele Vinci (*President of MASMEC SpA, Italy*)

Giuseppe Visaggio (*President for the Scientific Committee, DAISY-Net, Italy*)