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.
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; MEGA, the Middle Eastern 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).

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
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.
**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 Farmindustria
Michal 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

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Donato Bonifazi, Corporate Secretary Gianni Benzi Foundation - secretary@benzifoundation.org
13.30 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
G. Cessak, Polish Medicinal Agency
P. Siviero, Italian Medicinal Agency

Pharmaceutical Industries Representatives
G. Caruso, Farmindustria, 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

15.30 Coffee break

16.00 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
V. De Cian, Chiesi Farmaceutici

Paediatric Priority List
K. Connolly, European Medicines Agency; Portiuncula Hospital, Ireland

Ethical Risk/ Benefit evaluation
A. Altavilla, Université de la Méditerranée Marseille

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

J ouvenile animal studies to improve B/ R ratio
J. Gallego, 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
10.45 Coffee break

11.00 **Session 3**
Risk/ Benefit in the Committees evaluation process
Chairs: K.Connolly - W. Bianchi

PDCO Assessment of the B/ R ratio
D. Brasseur, European Medicines Agency

COMP Assessment of the B/ R ratio
B. Dembowska - Baginska, European Medicines Agency; Instytut Pomnik Centrum Zdrowia Dziecka

CHMP Assessment
M. Pirozynski, European Medicines Agency

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
13.00 Lunch
14.00 **Session 4**
Driving incentives by making a good application
Chairs: D. Brasseur - E. Bosone

IMPD for Phase 1 and procedures in Italy vs EU
S. Paratore, Novartis Farma
L. Lambiase, Novartis Farma

Procedure for PIP Approval
R. Ancuceanu, PDCO 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

16.30 **Session 5**
Take advice to be successful
Chairs: D. Konstantinov - C. Walker

Scientific advice and PA: preparation of the application
M. Pirozynski, European Medicines Agency

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
08.30 **Session 6** - B/R Assessment after the MA
Chairs: M. Migdal – A. Saint Raymond

Risk Management Plan in the B/R ratio definition
A. Castot, Agence Française de Sécurité Sanitaire

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

11.00 Coffee break

11.30 **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
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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:

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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)
100 km to Park Inn Hotel in Krakow from Airport Pyrzowice in Katowice (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 )

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 Przedsobiorstwo 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

- Minibus Company Carpolonia run on routes from the airport to Krakow www.carpolonia.pl
- Matuszek Buses run on routes: Krakow – Katowice www.matuszek.com.pl

Course Location

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