ISTITUTO DI RICERCHE FARMACOLOGICHE MARIO NEGRI

CENTRO DI RICERCHE CLINICHE PER LE MALATTIE RARE
ALDO E CELE DACCO'

Villa Camozzi, 24020 Ranica - Bergamo - Italy

July 10, 2003
14.00-18.00

European Symposium

Methodological aspects of clinical trials in rare diseases

The EUROPEAN SCHOOL OF RARE DISEASES has been established as an activity of the Clinical Research Center for Rare Diseases *Aldo e Cele Daccò*. Seminars, workshops, and courses are regularly scheduled at the School; meeting among physicians and patients with specific rare diseases have been also held.

The European Commission is supporting the activities of the School with a cycle of conferences and symposia which will cover several topics such as:
"The role of patient support groups for rare diseases". "Methodology for clinical research on rare diseases", "Priorities in research and development of orphan drugs", "Organization and implementation of Database Registries for Rare Diseases" "State of the art of gene therapy for rare diseases", "Socioeconomic burden of a rare disease on the families".

This program will be carried on with the collaboration of UNIAMO, the Italian Association for rare diseases; Accademia Nazionale di Medicina; AGRENSKA, Swedish Center for the families of children with rare disabilities, Centro de Investigacion sobre el Sindrome del Aceite Toxico, Centros Nacionales de Salud Pública, Madrid and the Spanish Federation of Rare Diseases.

The second symposium of the series is:
Methodological aspects of clinical trials in rare diseases
which is aimed to discuss methodological issues in designing and conducting clinical studies in small populations.

The Symposium will take place at Clinical Research Center for Rare Diseases *Aldo e Cele Daccò*, which is located in Ranica, near Bergamo Italy.

European Symposium

Program

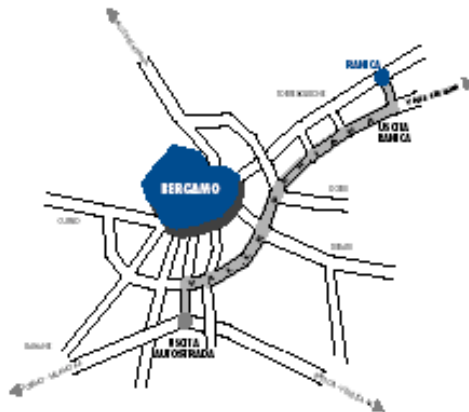|	
13.00	Registration
14.00	Welcome address **Giuseppe Remuzzi, Mario Negri Institute, Bergamo, Italy**
	Clinical trials: the Gold Standard **Brendan M. Buckely, University College, Cork, Ireland**
	Practical limitations of classical methodology in small population **Antoine Flahault, University Pierre and Marie Curie, Paris, France**
	Bayesian methods **Guido Bertolini, Mario Negri Insitute, Ranica, Italy**
	Quality of published randomized controlled trials in rare diseases **Annalisa Perna, Mario Negri Institute, Ranica, Italy**
17.00	Discussion

For Information

Scientific Secretary:	Arrigo Schieppati, Erica Daina
Organizing Secretary:	Daniela Riceputi, Monica Lena
Address:	Clinical Research Center for Rare Disease *Aldo e Cele Daccò* Villa Camozzi - Via G.B. Camozzi,3 - Ranica Italy tel. 0039 035 4535 314 - 0039 035 4535 304 fax. 0039 035 4535 370 email: riceputi@marionegri.it - raredis@marionegri.it
Registration:	For Registration please use the attached form and send it to Secretariat by June 30th 2003

The Clinical Research Center for Rare Diseases *Aldo e Cele Daccò* is located in Ranica, approximately 5 km from Bergamo. You can reach it by:
Car: leave the motorway A4 Milano-Venezia at the Bergamo exit. Follow the signs for "Valle Seriana" until you reach the turn off to Ranica, in center of Ranica you will see the road signs for Villa Camozzi.
Train/bus: if you arrive by train, take bus n. 1 directly outside the station, and get off at the second stop. Cross Viale Papa Giovanni XXIII and take bus n. 11/A in Via Tiraboschi. Get off at the terminus in Ranica and follow the road signs for Villa Camozzi

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name (first/middle/last)
nome

mailing address
indirizzo

city/state/zip
cap/città/provincia

hospital
ospedale

institution
istituto/ente

tel

fax

email

date/data   signature/firma