

## 3<sup>rd</sup> International Summer School

on

### Rare Disease and Orphan Drug Registries

21-23 September 2015, Rome (Italy)

#### Monday, 21 September

- 08:30 *Registration of the participants*
- 09:00 Presentation of the course  
**D. Taruscio**
- 09:15 Introduction to PBL  
**G. De Virgilio**
- 09:30 Working in small groups with facilitators:  
Problem analysis (*I session*)
- 11:00 *Coffee Break*
- 11:15 Working in small groups with facilitators:  
Problem analysis (*II session*)
- 12:30 Introduction to learning resources  
**L. Vittozzi**
- 13:00 *Lunch*
- 14:00 Individual work session
- 16:30 Monitoring meeting with facilitators
- 17:00 *End of the day*

#### Tuesday, 22 September

- 09:00 Registry feasibility and sustainability  
**S. Lynn**
- 09:45 Discussion
- 09:55 Registry scope and adaptation  
**J. Giuliano**
- 10:40 Discussion
- 10:50 *Coffee Break*

- 11:10 Registry governance  
**F. De Angelis**
- 11:55 Discussion
- 12:05 Registry interoperability and standardization  
**D. Alexandre**
- 12:50 Discussion
- 13:00 *Lunch*
- 14:00 Quality assurance  
**L. Palmieri**
- 14:45 Discussion
- 14:55 Data elaboration essentials  
**M. Santoro**
- 15:40 Discussion
- 15:50 Patients' needs and strengths  
regarding registries  
**R. Barbon Galluppi**
- 16:35 Discussion
- 16:45 *End of the day*

#### Wednesday 23 September

- 09:00 Working in small groups with facilitators:  
problem solution (*I session*)
- 10:30 *Coffee Break*
- 10:45 Working in small groups with facilitators:  
problem solution (*II session*)
- 11:30 Presentation of group solutions and feedback  
from peers and experts
- 13:00 *Lunch*
- 14:00 Experiences with building and managing a  
registry  
**W. Barcellini, P. Torreri**
- 15:00 Satisfaction questionnaire
- 15:30 Concluding remarks
- 16:00 *End of the course*

#### LECTURERS

- Diego Alexandre**, Institute of Medical Biostatistics, Epidemiology and Informatics, University Medical Center, Johannes Gutenberg University, Mainz, Germany
- Renza Barbon Galluppi**, UNIAMO - Federazione Italiana Malattie Rare Onlus, Venice, Italy
- Wilma Barcellini**, Fondazione IRCCS Ca' Granda, Ospedale Maggiore Policlinico, Milan, Italy
- Fernanda De Angelis**, PARENT Project Italia, Rome, Italy
- Giovanni De Virgilio**, External Relations Office, Istituto Superiore di Sanità, Roma, Italy
- Joseph Giuliano**, CHDI Foundation, Princeton, USA
- Stephen Lynn**, Institute of Human Genetics, Newcastle University, Newcastle upon Tyne, United Kingdom
- Luigi Palmieri**, National Center for Epidemiology, Surveillance and Health Promotion, Istituto Superiore di Sanità, Rome, Italy
- Michele Santoro**, Institute of Clinical Physiology, National Council of Research, University of Pisa, Pisa, Italy.
- Domenica Taruscio**, National Centre for Rare Diseases, Istituto Superiore di Sanità, Rome, Italy
- Paola Torreri**, National Centre for Rare Diseases, Istituto Superiore di Sanità, Rome, Italy
- Luciano Vittozzi**, past research director at National Centre for Rare Diseases, Istituto Superiore di Sanità, Rome, Italy

#### PBL FACILITATORS

- Daniela Coclite**, External Relations Office, Istituto Superiore di Sanità, Rome, Italy
- Alice Fauci**, External Relations Office, Istituto Superiore di Sanità, Rome, Italy
- Alfonso Mazzaccara**, External Relations Office, Istituto Superiore di Sanità, Rome, Italy
- Antonello Napolitano**, External Relations Office, Istituto Superiore di Sanità, Rome, Italy

## COORDINATORS AND SECRETARIAT

### Course Director

**Domenica Taruscio**

Director, National Centre for Rare Diseases  
Istituto Superiore di Sanità, Roma, Italy

### Didactic Coordinators

**Giovanni De Virgilio**

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## GENERAL INFORMATION

### Objectives of the Course

The course will enable the participants to establish a patient registry for clinical research on rare diseases and related treatments.

In particular, at the end of the course the participants will be able to:

- Describe the resources needed for the establishment of a clinical research registry;
- Describe the features of successful strategies to ensure long time sustainability of the registry, including registry purposes, data use, data sharing, dissemination activities and the role of patient associations;
- Describe the main steps for planning of a registry and keeping control on its operation.

### Learning method

The school will apply the Problem-Based Learning (PBL) methodology. PBL uses a problem as a didactic stimulus. Learning occurs by working at the explanation or solution of the problem in small groups assisted by a facilitator. The problem is presented at the beginning of the learning process. PBL is typically implemented in seven steps, as follows: 1) clarification of terms/concepts; 2) definition of the core issue/s; 3) analysis of the problem (formulation of hypotheses of problem causes in a brain storming fashion); 4) categorisation of hypotheses; 5) formulation of learning objectives needed to explain/solve the problem; 6) individual study (lectures, documents); and 7) problem solution.

**Language:** English

## ASSESSMENT OF THE ACHIEVEMENT OF THE LEARNING OBJECTIVES

The participants' performance will be mainly evaluated on the basis of the group work written solutions. No report card will be issued.

## ATTENDANCE CERTIFICATES

At the end of the course a certificate of attendance will be handed to the participants who attended at least 80% of the program. No credits of Continuing Education in Medicine will be issued.



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21-23 September 2015

*organized by*



National Centre for Rare Diseases, Istituto Superiore di Sanità

*in collaboration with*

External Relations Office, Istituto Superiore di Sanità

**Venue**  
Roma Scout Center  
Largo dello Scautismo, 1  
**Rome, Italy**