

3rd International Summer School on Rare Disease and Orphan Drug Registries

Start: September 21, 2015 at 8:30 – **End:** September 23, 2015 at 16:30

Venue: Aula Marotta, Istituto Superiore di Sanità, Via Regina Elena 299, Roma

DETAILED PROVISIONAL PROGRAM

Monday 21, September

- 08.30 Registration of the participants
- 09.00 Presentation of the course
D. Taruscio
- 9:15 Introduction to PBL
G. De Virgilio
- 9:30 Working in small groups with facilitators:
Problem analysis
Coffee Break (15 min)
- 12:30 Introduction to learning resources
L. Vittozzi
- 13:00 Lunch
- 14:00 Individual work session
- 16:00 Monitoring meeting with facilitators
- 17:00 End of the session

Tuesday 22 September

- 9:00 Registry feasibility and sustainability
S. Lynn
- 9:45 Discussion
- 9:55 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
F. Ückert
- 12:50 Discussion
- 13:00 Lunch Break
- 14:00 Quality assurance
S. Giampaoli
- 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
Working coffee break
11:30 Presentation of group solutions and feedback from peers and experts
13:00 Lunch break
14:00 Experiences with building and managing a registry
P. Torreri
15:30 Satisfaction questionnaire
16:00 Concluding remarks
16:30 End of the course

Lecturers and Facilitators

Renza Barbon Galluppi, UNIAMO, Italy
Fernanda De Angelis, PARENT PROJECT Italia, Italy
Giovanni De Virgilio, URE-ISS, Roma, Italy
Simona Giampaoli, ISS, Rome, Italy
Joseph Giuliano, CHDI Foundation, Princeton, USA
Stephen Lynn, University of Newcastle, UK
Michele Santoro, IFC-CNR, Pisa, Italy
Paola Torreri, CNMR-ISS, Rome, Italy
Frank Ückert, IMBEI, University Medical Center, Mainz, Germany
Domenica Taruscio, CNMR-ISS, Roma, Italy
Luciano Vittozzi, past research director at CNMR-ISS, Italy
Daniela Coclite
Alice Fauci
Alfonso Mazzaccara
Antonello Napoletano, URE – ISS, Roma

Course Director

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

Didactic Coordinator

G. De Virgilio
Director, External Relations Office
Istituto Superiore di Sanità, Roma, Italy

General information

General learning objective

To train participants on the establishment of a patient registry for clinical research on rare diseases and related treatments

Specific learning objectives:

- To inform on the resources available for the establishment of a clinical research registry
- To train on the implementation of successful strategies to ensure long time sustainability of the registry, including registry purposes, data use, data sharing and dissemination activities
- To train on the planning of a registry and keeping control on its operation

Learning method:

The school will apply the Problem-Based Learning (PBL) methodology. Problem-based learning (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 steps¹, 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

Participant selection procedure

Applications will be selected on the basis of the past experience and concrete interest and responsibility in the development and management of patient registries. Applicants will be informed by e-mail of the selection results.

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.

¹ Schmidt HG. The rationale behind problem-based learning. In: HG Schmidt, M Lipkin Jr, MW de Vries, JM Greep (Eds.). New directions for medical education. Problem-based learning and community-oriented medical education. 1989; 105-111.