

## Language

The official language will be English.

## Methods of Assessment

There will be no formal examination. A Certificate of Attendance will be awarded only to those completing the full course.

## How much will it cost?

The course is available free of charge. Please take note that the Istituto Superiore di Sanità will provide refreshments (tea, coffee and biscuits) and lunch but will not pay for travel and accommodation expenses.

## How to apply

The application will be available at: [www.iss.it/cnmr](http://www.iss.it/cnmr)

For more information visit the website  
[www.iss.it/cnmr](http://www.iss.it/cnmr)



### Course Director

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## International Summer School RARE DISEASE AND ORPHAN DRUG REGISTRIES

September 16-20, 2013

### *Preliminary Program*

Istituto Superiore di Sanità  
Aula Marotta  
Viale Regina Elena, 299  
Rome (Italy)

Organised by the  
National Centre for Rare Diseases  
Istituto Superiore di Sanità



### Monday, 16 September

8.30	<i>Registration and pre-test</i>
9.00	<i>Welcome and presentation of the course objectives</i>
9.30	<i>The role of registries in epidemiological, clinical and genetic research on rare diseases: overview and case examples</i>
10.30	<i>Patient driven registries</i>
11.00	Break
11.30	<i>Defining the aim(s) of a registry and addressing study design, objectives and methods</i>
13.00	Lunch
14.00	Small group exercise
17.00	Adjourn

### Tuesday, 17 September

9.30	<i>Defining the data elements</i>
11.30	Break
12.00	<i>Selecting data-sources and variables</i>
13.00	Lunch
14.00	Small group exercise
16.00	Adjourn

### Wednesday, 18 September

9.30	<i>Quality assurance</i>
11.30	Break
12.00	<i>Steps for assuring quality of a registry</i>
13.00	Lunch
14.00	Small group exercise
16.00	Adjourn

### Thursday, 19 September

9.00	<i>Ethical and legal issues -data protection directive and the ethics of solidarity</i>
10.30	Break
11.00	<i>Adaptive governance for ensuring transparency</i>
13.00	Lunch
14.00	Small group exercise
16.00	Adjourn

### Friday, 20 September

9.30	<i>Registry sustainability: funding, operation, termination</i>
11.00	Break
11.30	<i>Focus groups: The culture of Data Sharing</i>
13.00	Lunch
14.00	<i>Evaluation questionnaire and post-test</i>
14.45	<i>Certificate delivery and greetings</i>
15.00	<i>End of course</i>

### Description

The course will take the participants through the main concepts and practical steps that must be undertaken in the establishment and management of a rare disease registry to ensure its usefulness, soundness and sustainability. The course will provide basic notions on the methodology of observational studies with a view to the specificity of rare disease registries, on the selection of data elements with a focus on the interoperability of rare disease registries, on quality assurance and on the technical and legal tools that must be adopted to protect patients' data confidentiality. The course will consist of frontal presentations followed by small group exercises.

### Important dates

**24 April 2013 – application is open**  
**30 June 2013 – deadline for application submission**  
**10 July 2013 – confirmation of admittance**  
**16-20 September 2013 – course**

### Eligibility requirements

The course is open to the following profiles:

- professionals involved in the health care of rare disease patients;
- professionals working in public health institutions in the surveillance of rare diseases;
- persons working or volunteering for a patients' association;
- professionals working in the pharmaceutical industry and especially in the development or surveillance of orphan medicinal products.

Priority will be given to participants:

- 1) already involved in the conduction and management of a rare disease registry or patient data collection;
- 2) with a concrete intention to establish a new registry for a rare disease.