

International School of Public Health on Rare Diseases

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To improve knowledge for the majority of rare diseases
To increase the capacity of public health intervention
a) in many countries; b) country /Region specificity

- Prevention
- Diagnosis
- Treatments
- Rehabilitation

- Social and health services
- Health care
- Education / training courses

- Research
- Natural history of diseases
- Etc.



A) Situation analysis of a Country

B) Tailored training courses for professionals and policy makers on

- Country specific needs
- emerging national issues

The courses will encompass

- the evidence-based characterization of risk factors
- genetic counseling
- running and use of registries
- epidemiological studies
- risk communication
- ethical and legal issues
- Privacy
- Data sharing

Training courses on

Prevention (I, II and III)
in collaboration with EUROCAT and /or
Clearinghouse for the prevention of congenital
anomalies

Epidemiology of rare diseases (e.g. registries)

Health care guidelines

Towards a diagnosis

Experiences at the Istituto Superiore di Sanità since several years

- International Summer Schools
- Training Courses

Registries

Guidelines

Diagnostic suspicion

Quality assurance of genetic testing

Primary prevention of congenital anomalies

Secondary prevention of rare metabolic diseases



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II INTERNATIONAL SUMMER SCHOOL - RARE DISEASE AND ORPHAN DRUG REGISTRIES

Organized by the Istituto Superiore di Sanità, Rome (Italy), September 15-19, 2014

Endorsed by ICORD (www.icord.se)

See below the Provisional Program

Patient registries are key tools in rare disease research and are now the subject of unprecedented popularity in rare disease (RD) translational research and of extensive policy actions. The establishment and maintenance of a successful registry is a scientific and managerial challenge. Therefore, it is important that the methodological approaches, experiences and lessons learned in the development of a registry are shared.

The School is open to researchers, medical specialists, experts in statistics, health authorities, academia, medical students and patient organizations, who are involved or intend to establish a rare disease patient registry.

The School will take the participants through the main concepts and practical steps that must be undertaken in the establishment and management of a RD patient registry in line with the present challenges posed by new technologies, new data collection and analysis methods and new registry applications.



Intl Summer School Rare Diseases & Orphan Drug Registries. Rome (Italy), September 15-19, 2014





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HEALTH CARE GUIDELINES FOR RARE DISEASES

Organized by the Istituto Superiore di Sanità, Rome (Italy)

Endorsed by ICORD: www.icord.se

1) International Course "Quality assessment of health care guidelines for rare diseases"

When: January 26-27, 2015

Where: Istituto Superiore di Sanità, Rome (Italy)

Target: Health professionals

Language: The official language will be English

The course is free of charge