Health literacy and health education fostering participation and improving women’s and men’s health
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Training Program on Orphan Drugs Targeting Patients’ Associations and Families of Patients with Rare Diseases

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The Italian Background

- In very recent years increased acknowledgement of the importance of the active involvement of patients
- Patient and public engagement in determining priorities for service development could help to ensure that policy-making keeps in tune with population requirements
- Association of patients with rare diseases can play an active role for improving quality of care
- Initiatives or training courses addressed to patients and more specifically to patients with rare diseases are few

The Italian National Center for Rare Diseases

National Center for Rare Diseases

Person in charge: Domenica Taruscio

Rare Diseases

What are Rare Diseases?
A disease is considered rare when it affects 1 individual among 2 thousands persons. There are from 5 thousands to 8 thousands different pathologies that only in Europe affect more than 15 million inhabitants.

Rare Diseases exempt from contribution in Italy
Ordered list of pathologies and online search.

I have a Rare Disease? What do I have to do?
There are several steps to be followed to understand the world of rare diseases: from the diagnosis to the care centres and the Associations of patients.

News

February 29, 2012 - Rare disease day
The events organised by the Italian National Centre for Rare Diseases

Other Sites

- European projects
- Genetic Testing
- Guidelines
- Italian Network for Folic Acid Promotion
- Narrative Medicine
- Orphan Drugs

Registries

National Registry of Rare Diseases

National Registry of Orphan Drugs

Congenital Malformations Registers

Rare Diseases

A large group of diseases which are characterized by a low prevalence in the population

In Europe a disease is considered rare when it affects 1 individual among 2 thousands

More than 15 million inhabitants in Europe affected by rare diseases

Orphan Drugs

Product that is potentially useful in treating a rare disease but does not have a market sufficient to cover the costs of its development

The pharmaceutical industries are not interested in investing in a product which serves only a few patients

The medicinal product is therefore without a sponsor, or orphan

Training program on orphan drugs for rare diseases

- The Italian National Institute of Health (ISS) developed an educational project about Orphan Drugs
- Addressed to the members of the Associations of patients and relatives of patients with rare diseases
- The project was financed by the Italian Agency for Drugs
- 2007-2010

Project Objectives

- To increase Associations knowledge on orphan drugs:
  - drugs development, regulatory process, clinical trials, ethical issues
- To identify specific information/educational needs of patients' Associations
- To develop a training curriculum on orphan drugs on the basis of the information/educational needs assessment

Methodology - 1

- Assessment of information/training needs of Associations
- Questionnaire sent to 253 associations
- The questionnaire was developed on the basis of a bibliographic review of literature on the needs of patients with rare diseases
- It included questions about the structure of the association, their activity on orphan drugs and their opinions on the quality of the information available on orphan drugs

Methodology - 2

- 102 associations (40.3%) filled and returned the questionnaire (from June to October 2008)
- The response rate was almost identical in the three geographical areas (North – Center - South) of our country
- Questionnaires results were integrated with
  - Information on patient’s needs deriving from the Rare Diseases Center toll free number service
  - Patients experience - Narrative Medicine section of the website

Information Required by Associations

- Drug development process
- Regulatory process for orphan drugs including reimbursement procedures
- Development and availability of guidelines for rare diseases
- Information sources for rare diseases
- Innovative medicines

Training program steps

- Development of the training courses on the basis of needs assessment
- Implementation of the training program through residential meetings
- Cascade training ⇨ Training of trainers
- Implementation of trainers activities within each Association
- Final workshop to present the activities performed by each trainer.

Training courses

Training courses held in the following sites:

1. National Institute of Health in Rome (for Central Italy)
2. Documentation Center of the Regional Hospital Santa Maria degli Angeli - Pordenone (for Northeast Italy)
3. Department of Public Health and Microbiology University of Turin (for Northwest Italy)
4. Centre for Training and Research in Public Health of Caltanissetta (for South and Insular Italy)

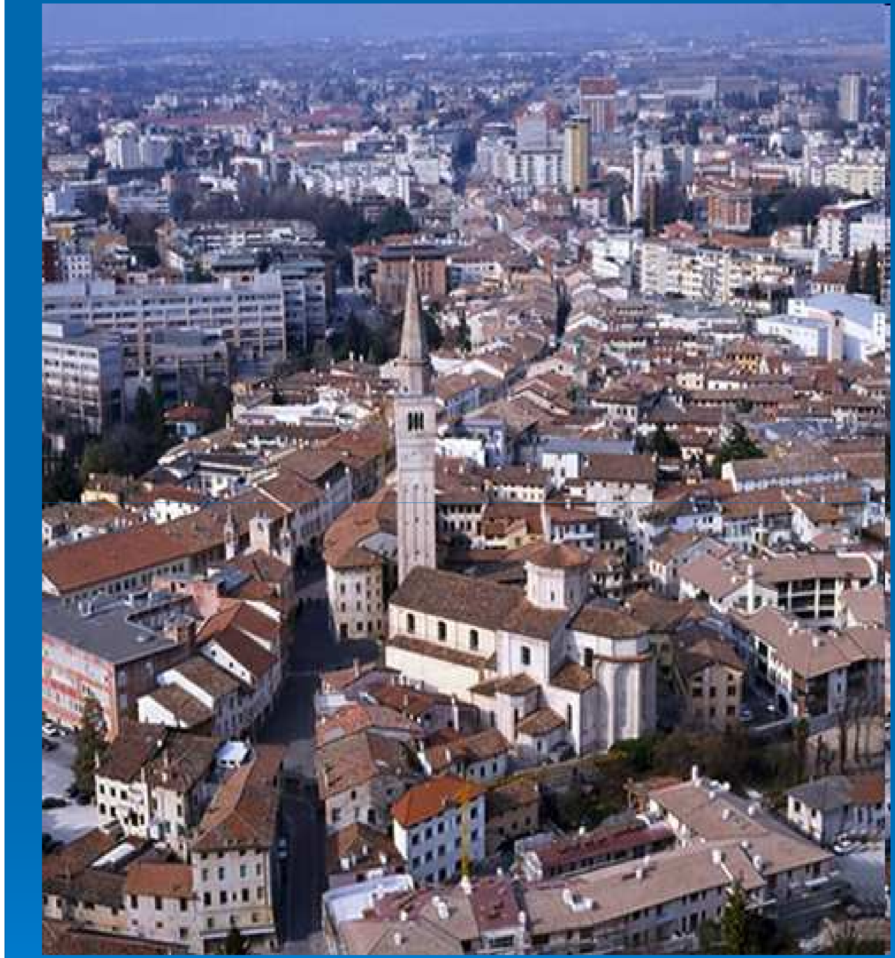
Four Italian Areas

First training course - Center

Rome

National Institute of Health

Second training course - NE



Pordenone

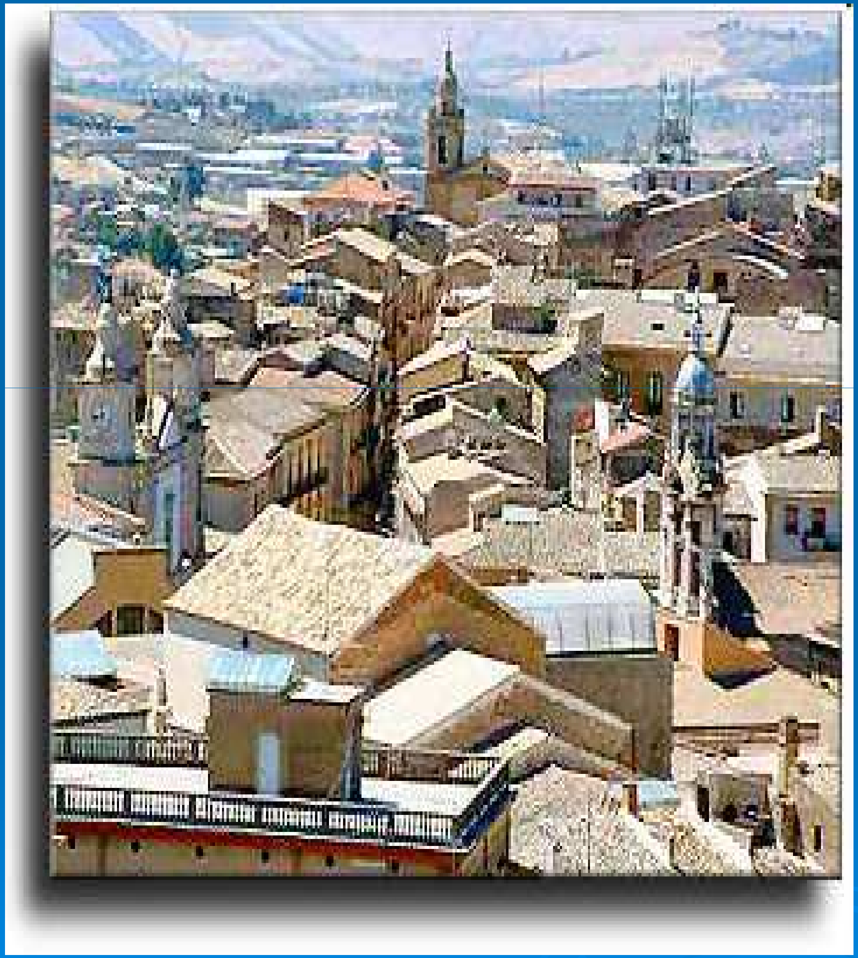
Hospital Santa Maria degli Angeli

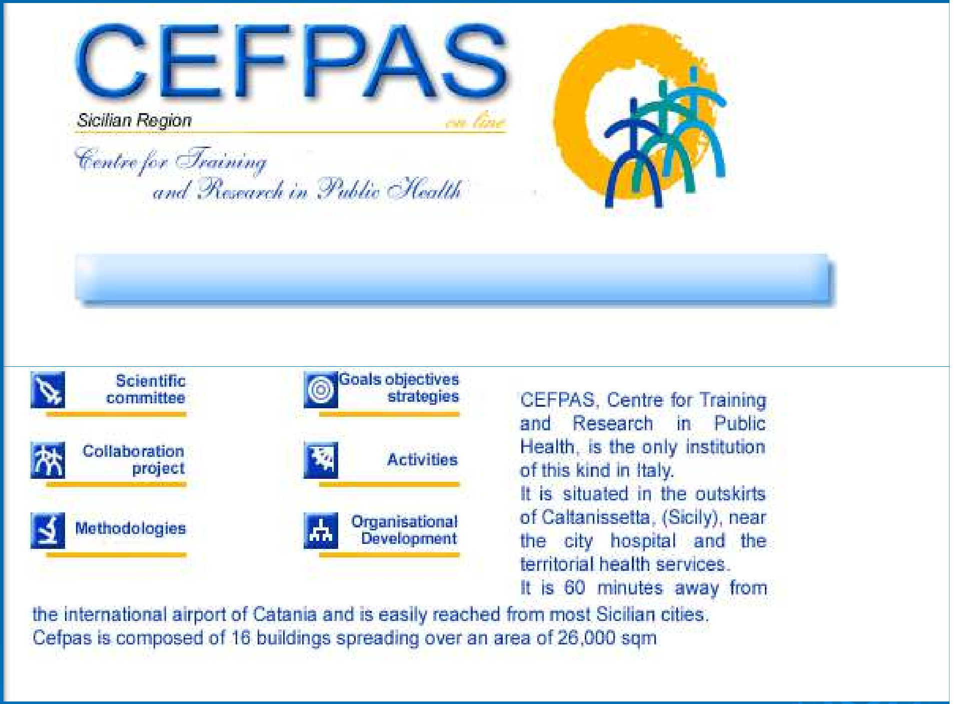
Third training course - NW

Turin

University of Turin
Department of Public Health

Fourth Training Course -South





CEFPAS, Centre for Training and Research in Public Health, is the only institution of this kind in Italy.
It is situated in the outskirts of Caltanissetta, (Sicily), near the city hospital and the territorial health services.
It is 60 minutes away from the international airport of Catania and is easily reached from most Sicilian cities.
Cefpas is composed of 16 buildings spreading over an area of 26,000 sqm

Center for Training
and Research
in Public Health

Caltanissetta, Sicily

Training Course Contents

- Development and analysis of guidelines for specific diseases
- Ethical aspects of scientific research
- Databases, Registries, Cohorts of patients
- Clinical Research and special use of drugs
- New drugs development: laboratory research, clinical trials and post marketing
- Information Retrieval and tools for Web searching

Practical Work during course

- Small groups with tutor
- Fieldwork
- Presentation of Project Work
- Collaboration Strategies between Patient Associations and National Center for Rare Diseases

Weakness of the programme

- Up to 120 participants could be admitted to the courses (30 per course)
- Low participation of the representatives of patients associations due to:
  - Difficulty to leave the job and the family for several consecutive days
  - Other tasks required to associations (conferences, workshops, meetings)
  - Cost for the participation in the courses not totally reimbursed (up to € 250)

Evaluation questionnaire
Score 1-5

- *The objectives of the course were clear 4.4*
- *The level of lessons was appropriate to my knowledge 4.3*
- *The training course was well organized 4.4*
- *The teaching methodology was clear 4.6*
- *I learned new concepts 4.6*
- *I learned new skills 4.3*
- *The quality of didactic material was appropriate 3.7*
- *The number of teachers was too low 2.0*
- *The time dedicated to the lessons was too much 2.1*
- *The content of the course was in line with its objectives 4.4*
- *I can apply what I learned during this course in my job 4.1*
- *The course is too short to meet the objectives 2.9*
- *The didactic material is not enough 2.2*

Positive aspects of the training course

- Openness to dialog
- Possibility to know the different local situations of Associations working with rare diseases
- Possibility to exchange experiences among peers
- High skills of the teachers
- Training method used