

# Italian Network for Rare Diseases : legislation and organization

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# RARE DISEASES : PUBLIC HEALTH INITIATIVES

Europe

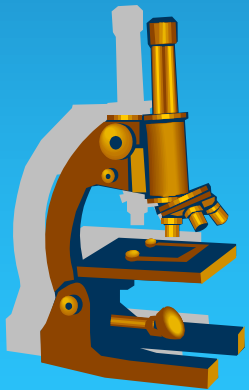
Italy



# REGULATIONS ON RARE DISEASES IN ITALY



- 2 National Health Plans '98-00; 2003-05
- Regional Health Plans
- National Network for Rare Diseases (2001-)
- Agreement between the Ministry of Health and Regions (2002-)
- National Committee on RD
- Research Projects for RD
- Research Plans for Orphan Drugs (AIFA)



# Italian National Health Plans 1998-2000; 2003-2005

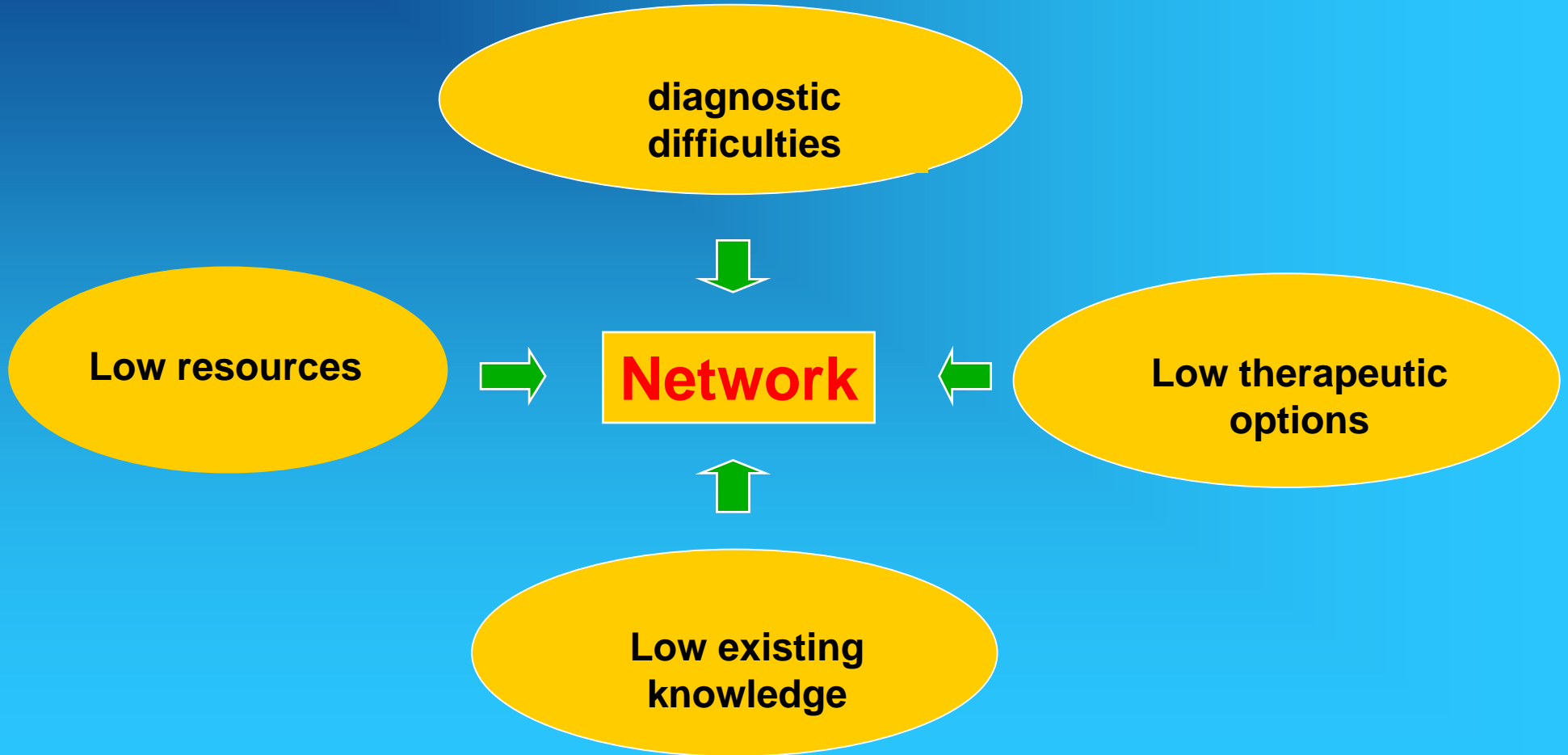
## National Health Plans

underline the importance of:

- scientific research on rare diseases
- prevention activities
- appropriate and timely diagnosis
- treatment for patients with RD

# TO IMPROVE COLLABORATION

## FOR RARE DISEASES...



# **NATIONAL NETWORK FOR RARE DISEASES** **(Govenmental Decree 279/2001)**

**It's a network for**

**Prevention  
Surveillance  
Diagnosis  
Treatment**

**National Registry Rare Diseases  
(Istituto Superiore di Sanità)**

**about 500 Rare Diseases (284 specific RD+ 47 groups of RD):  
health care services are provided at no charge to patients**

# Rare diseases: criteria

- *rarity* - prevalence according to UE definition (<5 /10.000 inhabitants)
- *clinical seriousness of the RD*
- *degree of disability*
- *cost of treatment*

# NATIONAL NETWORK FOR RARE DISEASES

*set up in order to :*

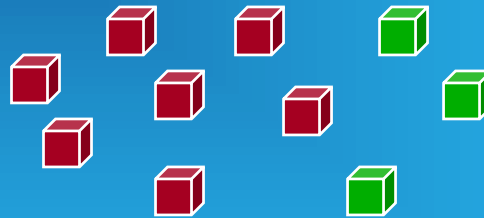
- **Implement prevention activities**
- **Develop epidemiological surveillance**
- **Implement both diagnosis and care intervention**
- **Improve health operators training**
- **Promote citizens information**

# Network for health services and epidemiological flow

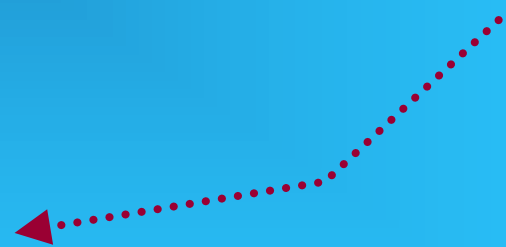
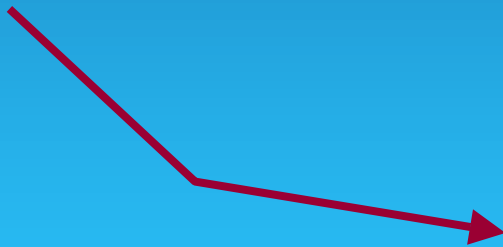
REGIONS



Regional Centre



Inter-regional  
*Coordination*  
Centre



National registry of rare diseases  
Istituto Superiore di Sanità (Rome)

# **NATIONAL NETWORK FOR RARE DISEASES**



## **National Registry of Rare Diseases**

### **General objectives**

- A) National and regional health planning**
- B) Surveillance of rare diseases**

# NATIONAL REGISTRY OF RARE DISEASES

## Specific objectives

To estimate incidence or/and prevalence

To define standartized diagnostic and therapeutic protocols

To improve collaboration among health care operators

# Italian Regions (brown) which have identified Centres for diagnosis and treatment of rare diseases

*Presidi individuati da Delibera di Giunta Regionale, ufficialmente comunicati dalle Regioni al Ministero della Salute e all'Istituto Superiore di Sanità (D.M.18 maggio 2001 n. 279)*



# CRITERIA

## adopted by Regions to select Centres

- General settlement by the Decree (279/2001): documented specific experience in diagnostic or therapeutic activities and required health care facilities
- more specific: scientific publications /impact factor, collaboration with local health services, consulting to GPs, easy access to services
- uniform coverage of the territory

# Who performed the selection ?

*In most cases:*

- *Ad hoc* Technical Organism (Technical-Scientific Committee)
- National Agencies for Regional Health Care Services (ARS/ARES)

# HOW REGIONS PERFORMED THE SELECTION ?

- Every hospital declared its own experience by answering to a questionnaire.
- Regions verify the structures competences by processing activities data and analyzing the admission rates to the hospital care services.

# ITALIAN REGIONAL NETWORKS FOR RARE DISEASES

*Structures are selected according to their competences in :*

- ❖ Both Health care and Diagnostic services
- ❖ Health care services mainly
- ❖ Exclusively diagnostic services
- ❖ Every single RD or groups of RDs
- ❖ All RDs or great categories

# **ACTIVITIES OF REGIONAL HOSPITALS / CENTRES**

**according Article 2 (Decree 279 / 2001):**

- Behave in accordance with clinical protocols drawn up by Interregional / National Reference Centres**
- Collaborate with GPs and territorial health care/social Services**

# ACTIVITIES OF INTERREGIONAL REFERENCES

## CENTRES :

- **Maintaining local epidemiological registers and send the epidemiological flow to the National Registry of RD**
- **Sharing information with other Reference Centres and with international organisms**
- **Co-ordinating regional hospitals by means of clinical protocols, guidelines, etc.**
- **Supporting and guiding GPs and NHS physicians**
- **Training activities directed to health operators**
- **Providing information to patients'Associations**

# Italian Constitution

Law N. 3 (Official Journal-G.U. N. 248, 24.10.2001 2001):

Health Care is matter on which Central Government and Regions share their competences:

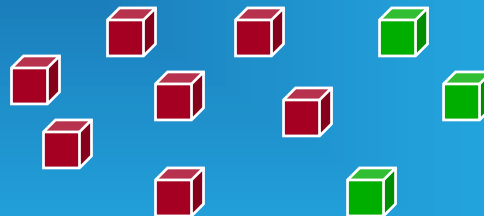
- Central Government states the main guiding principles by law;
- Regions define how health care services are delivered (number of services, number of health operators, etc.)
- Essential Health Care levels (LEA) are established by Central Government by laws and Regions are expected to comply with them.

# Network for health services and epidemiological flow

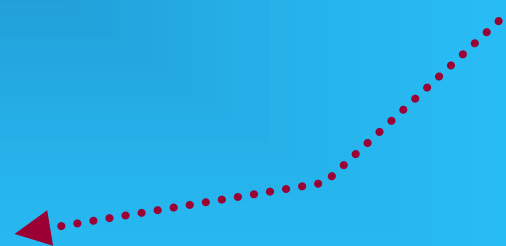
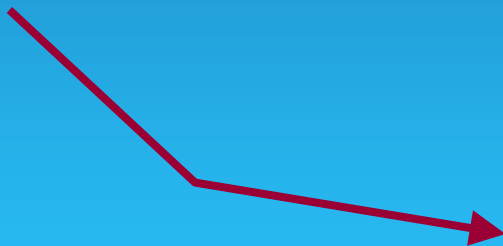
REGIONS



Regional Centre



Inter-regional  
*Coordination*  
Centre



National registry of rare diseases  
Istituto Superiore di Sanità

# Agreement between the Ministry of Health, Istituto Superiore di Sanità and all Regions (July 11, 2002)

- ✓ To co-ordinate the regional network activities
- ✓ To perform clinical protocols and guidelines
- ✓ To develop epidemiological surveillance
- ✓ To disseminate information on RD
- ✓ To collaborate with volunteers and non-government associations

# CRITICAL ASPECTS

- The Network is not complete (three Regions are still working)
- Criteria used by Regions to identify Centres are not homogeneous (e.g. documented experience in diagnostic or therapeutic activities; number of patients; required health care facilities)
- Each Region has adopted a different model to organize the regional network, therefore there are differences in the management of RD patients.
- The interregional referral centers have been not identified yet.
- Epidemiological flow is not mandatory.

**RESEARCH**

**NATIONAL  
REGISTRY  
RARE DIS.**

**NETWORK  
PEDIATRICS**

**E. Q. A.  
GENETIC  
TESTS**

**PATIENTS  
ASSOCIATIONS**

**NATIONAL CENTRE RARE DISEASES**  
<http://www.cnmr.iss.it>

**ISTITUTO SUPERIORE DI SANITA'**

**INFORMATION**

**ORPHAN  
DRUGS-  
EMEA**

**MEETINGS  
COURSES**

**NEPHIRD**

