

## National Plan for Rare Diseases Romania summary

**I.** Defining an institutional setting to assure the progress of the problems that rare diseases cause.

Activities:

1. Creating an institutional setting to assure the development of services to prevent diagnosis and rehabilitation of patients with rare diseases.

Founding **the National Committee for the Persons with Rare Diseases-NCPRD.**

**II.** The development of regional centers of reference – there are 3 regional centers within the system currently in 2008 concentrating on university centers, collaborating with centers of expertise, all taking place within the European Referencing system.

- plan to have 5 regional centers by 2013

### Aim of the referencing centers.

- To facilitate the diagnosis and to define a strategy of therapeutic and psychological care, also to help with social problems.
- To elaborate on how people are cared for with MS and ASP
- Epidemiological research and development.
- To provide training and information for the professionals working in this domain and also for patients and their families.
- Coordinating and providing medical and social care.
- To act as a mediator between ministers and the associations for patients.

### Evaluating the criteria for the nomination of centers.

- Activity (number of patients)
- Organizing the medical management process.
- Multi-disciplined, expertise
- Prescription and following of other expensive medical products
- Technological platform (highly specialized biological testing, molecular biology)
- Coordination of the system
- Informing/training (patients, help and care professionals about systems)

### Research

- Promoting
- Clinical projects and financed research
- Providing a good quality guide
- Looking after development

- Following epidemiology, to develop relevant indicators of health
- Following the development of activities

### **III Defining therapeutic, psychological and social strategy**

30.06.2008

- Creating recommended guide or protocol for care
- Diagnosis and promoting a good model of practice, following the patient and his family
- Collaboration of patients associations where there exists research and epidemiological surveillance
- Evaluating practices

**IV Permanent implementation of a national plan** in reference to people with rare genetic diseases in accordance with European Legislation and in partnership with EURODIS, the national alliance and consul. This is done through the Europlan project, financed by the European Commission/ DC Sanco, following the recommendation given by CE about rare diseases. A public debate was raised at the Lisbon Conference about the European Commission of rare disease/ announced under 14.02.2008.

### **V Creating a national register for rare diseases.**

**5.1 Founding the rare disease register**, epidemiological supervision organized for genetic rare disease through the current political sphere.

**5.2 Founding a register for people with rare disease, to be treated with Orfane medicine**

**5.3 Defining a communication strategy** between local, regional and national levels of responsibility.

- Structuring and coordination of medical and social care including health insurance, education and job location.
- Training and informing the health care professionals, patients and their families.

### **VI Developing good quality services for patients with rare diseases**

**6.1 Improving the access to information about rare disease**, sustaining the information centers, helplines and tel-verde.

To proceed with awareness campaigns to combat stereotyping and prejudice and to make them sensitive to the needs of people suffering from rare diseases; to sustain and be receptive to others rights.

Cooperation and financial support in organizing national campaigns for example

## **RARE DAY FOR RARE DISEASES**

29.02.2008 in years to come this day will be celebrated on 29<sup>th</sup> February.

**6.2 Developing preventative services for rare diseases** (i.e. implementing screening at a national level for phenylketonuria and hypothyroidism, and to improve access to genetical diagnosis services.

Extending groups for which screening is used for diagnosis.

**6.3 Developing continuous services, including rehabilitation** for patients for rare disease.

Founding and sustaining rehabilitation centers specialized in different disabilities produced by rare genetic diseases.

Sustaining activities, counseling and information by centers.

- Establishing protocol for diagnosis and care with experts' approval because of a lack of scientific proof
- Process of 12-18 months
- Creating emergency cards and personal care. The possibility of reimbursing treatments without market authorization; if the first treatment was given by the reference center.

## **VII Developing human resources**

**7.1 Training programs for specialists** from different rare disease domains.

Example- Cooperation in partnership with developing the human resources program with the minister of work, families and equality of opportunity.

PHARE program 2006- economical and social cohesion developing human resources, an irredeemable financial scheme-social services.

**7.2 Assuring personal specialist** hired to attend the people with rare disease in relation to all social and medical services; genetics, specialist doctors, biologists, medical assistants, laboratory workers, social assistants, physiologists, ergo therapy trainers, kinetotherapy, speech therapy, special needs teachers, support teachers, special educators.

- **training courses, adapting university curriculum.**

## **VIII Intensifying the efforts in favour of orphan drugs,**

**8.1 Assuring the availability of orphan drugs and compensating the cost/compensation of Orphan drugs** through the health insurance system.

- Evaluation of the added therapeutic value of all orphan drugs by the ministry of health and national agency for medicine.
- Production costs of orphan drugs will be supported in the social budget of social insurance from the budgets of hospitals or referencing centers.
- The establishment of protocols for diagnosis and care by the ministry of health and referencing centers. Financing without marketing authorization, nutritional supplements, creams and bandages, special dental care, clothes, (example for skin diseases) according to models of good practice and patients' needs.

**8.2 The supplementation of number and types included in health insurance,** contracts and the diversification of assistance devices, prosthetics, hearing aids, lenses, magnifying glass, devices, technical aid mobility devices.

## **IX Stimulating research in the domain of rare disease management**

### **9.1 Improving capacity to access research projects about rare disease utilizing international partnerships.**

- Course and instructions
- Joining networks in the European Countries particularly in epidemiology, diagnostics, information and research.

### **9.2 Stimulating scientific research, tackling rare diseases in Romania.**

Example- FP7 project etc. ( rare disease is a priority)

-strategic orientation for research

-addressing patients' needs other than medical care.