



EUROPLAN PROJECT NATIONAL CONFERENCE ON RARE DISEASES

WORKSHOP GATHERING THE EXPERTISE ON RARE DISEASES AT THE EUROPEAN LEVEL



**RELEVANT EXTRACT FROM THE
EUROPEAN COUNCIL RECOMMENDATIONS
ON ACTIONS IN THE FIELD OF RARE DISEASES**

LUXEMBOURG, 9 June 2009

COUNCIL RECOMMENDATIONS

“HEREBY RECOMMENDS that Member States:

Gather national expertise on rare diseases and support the pooling of that expertise with European counterparts in order to support:

- (a) the sharing of best practices on diagnostic tools and medical care as well as education and social care in the field of rare diseases;**
- (b) adequate education and training for all health professionals to make them aware of the existence of these diseases and of resources available for their care;**

COUNCIL RECOMMENDATIONS

- (c) the development of medical training in fields relevant to the diagnosis and management of rare diseases, such as genetics, immunology, neurology, oncology or paediatrics;**
- (d) the development of European guidelines on diagnostic tests or population screening, while respecting national decisions and competences;**
- (e) the sharing Member States' assessment reports on the therapeutic or clinical added value of orphan drugs at Community level where the relevant knowledge and expertise is gathered, in order to minimise delays in access to orphan drugs for rare disease patients.”**



**RELEVANT EXTRACT FROM THE
SPECIFIC EUROPLAN RECOMMENDATIONS
FOR THE DEVELOPMENT OF NATIONAL PLANS
FOR RARE DISEASES**

EUROPLAN RECOMMENDATIONS

- The use of international global information websites and data repositories for rare diseases is supported
- Help lines for health professionals are supported
- Awareness of the academy is raised on rare diseases, with particular attention to those lacking treatment
- Information for health professionals is made available for joining European Reference Networks
- The curriculum of the medical degree course includes an education package on rare diseases and on the relevant, specific provisions in the healthcare services
- Training of medical doctors, scientists and new healthcare professionals in the field of rare diseases is supported

EUROPLAN RECOMMENDATIONS

- Specifically targeted training to general practitioners is provided
- The exchange and sharing of expertise and knowledge between centres within the country and abroad is promoted
- Collaboration is ensured in the European evaluation of the existing screening programs
- The development and dissemination of evidence-based and internationally agreed guidelines and best practices on rare diseases is promoted
- An inventory of the accessibility to orphan drugs, including reimbursement issues, is implemented
- Dissemination of the information about orphan drugs, e.g. EMA decisions, is ensured in the most effective way, to avoid delays of the orphan drug accessibility

EUROPLAN RECOMMENDATIONS

- Provisions are set out for the off-label use of approved medicinal products for application to rare diseases; the use of drugs still under clinical trial is facilitated; measures of compassionate provision of orphan drugs are considered
- Participation is ensured in actions promoting the marketing authorisation of orphan drugs in all EU Countries; initiatives are taken for a common pricing mechanism
- Patients' access to treatment of authorised orphan drugs is recorded at national or EU level
- The list of on-going clinical trials on Orphan Medicinal Products included in the European database for clinical trials on Orphan Medicinal Products (EUDRA) is made public in order to improve quality of clinical trials through a higher number of patients and more reliable data



**RELEVANT EXTRACT FROM THE
EUROPLAN INDICATORS
TO EVALUATE THE ACHIEVEMENTS OF RD INITIATIVES**

EUROPLAN INDICATORS

ACTIONS	INDICATORS	TYPE	ANSWERS
Existence of a information sites for both professionals and patients provided by the plan/strategy	Existence of a comprehensive national and/or regional RD information system supported by the government	Process	Yes, covers most RD Yes, covers only some RD Not formal decisions have been taken
	Help lines for professionals	Process	Yes, covers most RD Yes, covers only some RD Not formal decisions have been taken
	Help lines for patients	Process	Yes No In process
	Clinical guidelines	Outcomes	Number ranging between 0 to 30

EUROPLAN INDICATORS

ACTIONS	INDICATORS	TYPE	ANSWERS
Promoting training activities and awareness educational campaigns among professionals and patients	Number of such as activities promoted by the plan/strategy	Process	Number ranging between 0 to 30



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FURTHER GUIDELINES FOR DISCUSSION

INVENTORIES, REGISTRIES AND LISTS

- Does your country participate to the development of a EU inventory of Rare Diseases as recommended in the Council Recommendations on rare diseases?

EU COLLABORATION ON RESEARCH ON RD

- How to foster and support the participation of national researchers and laboratories, patients and patients' organisations in EU-wide projects?

HOW TO SHORTEN THE ROUTE TO DIAGNOSIS

- How to organise DNA and samples exchanges and reimbursement at European and international level?
- How to support the development of European guidelines on diagnostic tests and population screening?
- What mechanisms to develop in order to support common protocols and recommendations such as European reference opinions on diagnostic tools, medical care, education and social care?

ACCESS OF RD PATIENTS TO ORPHAN DRUGS PRICING AND REIMBURSEMENT

- Participation to the EU-level collaboration on the assessment of the **Clinical Added Value of Orphan Drugs** at the European Medicines Agency (EMA).

HOW TO ENSURE ADEQUATE TRAINING OF HEALTHCARE PROFESSIONALS ON RARE DISEASES

- What mechanisms can be put in place to support the exchange of expertise at EU level and the adequate training for all healthcare professionals?
- Is your country supporting the participation of national experts in developing international guidelines to guide diagnosis and treatment of RD at national level?

SUPPORT TO THE ACTIVITIES PERFORMED BY PATIENT ORGANISATIONS

- What mechanisms can be put in place to support patients' empowerment activities and their representativeness in EU-wide instances?



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PROPOSALS FROM THE AUDIENCE