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;

EURORDIS
Rare Diseases Europe

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

- R5.1 The use of international global information websites and data repositories for rare diseases is promoted.
- R 5.2 Access to knowledge repositories and to expert advice for health professionals is established.
- R 5.3 Information on how to establish or join a European Reference Network is made available for health professionals.
- R5.4 The curriculum of the medical degree course includes an education package on rare diseases and on the relevant, specific provisions in the healthcare services.
- R5.5 Training of medical doctors (general practitioners and specialists), scientists and new healthcare professionals in the field of rare diseases is supported.

EUROPLAN RECOMMENDATIONS

- R 5.6 Continuing education programmes on rare diseases are made available for health professionals.
- R5.7 The exchange and sharing of expertise and knowledge between centres within the country and abroad is promoted.
- R5.8 Collaboration is ensured in the European evaluation of the existing screening programmes.
- R5.9 The development and adoption of good practice guidelines for rare diseases is promoted. The guidelines are made publicly available and disseminated as of the reach targeted health professionals.
- R 5.10 Dissemination of the information about treatment for rare diseases is ensured in the most effective way, to avoid delays of treatment accessibility.

EUROPLAN RECOMMENDATIONS

- R 5.11 Participation is ensured in common mechanisms, when available, defining conditions for the off-label use of approved medicinal products for application to rare diseases; for facilitating the use of drugs still under clinical trial; for compassionate provision of orphan drugs.

- R 5.12 An inventory of orphan drugs accessible at national level, including reimbursement status, is compiled and made publicly available.

- R 5.13 Patients' access to authorised treatment for rare disease including reimbursement status, is recorded at national and/or EU level.

EUROPLAN RECOMMENDATIONS

- R5.14 The list of ongoing clinical trials on Orphan Medicinal Products included in the European database for clinical trials on Orphan Medicinal Products (EUDRA) is made public at national level.

- R 5.15 All information on centres of expertise, good practice guidelines, medical laboratory activities, clinical trials, registries and availability of drugs, collected at national level, is also published on Orphanet as planned in the Joint Action.

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

EURORDIS
Rare Diseases Europe

WORKSHOP
GATHERING THE EXPERTISE ON RARE DISEASES AT THE EUROPEAN LEVEL

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?

EURORDIS
Rare Diseases Europe

STANDARDS OF CARE - CENTRES OF EXPERTISE

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?

EURORDIS
Rare Diseases Europe

WORKSHOP
GATHERING THE EXPERTISE ON RARE DISEASES AT THE EUROPEAN LEVEL

PROPOSALS FROM THE AUDIENCE