EURO PLAN
European Project for Rare Diseases
National Plans Development

EURORDIS
Rare Diseases Europe

EUROPLAN PROJECT

NATIONAL CONFERENCE ON RARE DISEASES

WORKSHOP
RESEARCH ON RARE DISEASES

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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:

- **Identify ongoing research and research resources in the national and Community frameworks in order to establish the state of the art, assess the research landscape in the area of rare diseases, and improve the coordination of Community, national and regional programmes for rare diseases research.**

- **Identify needs and priorities for basic, clinical, translational and social research in the field of rare diseases and modes of fostering them, and promote interdisciplinary cooperative approaches to be complementarily addressed through national and Community programmes.**

COUNCIL RECOMMENDATIONS

- Foster the participation of national researchers in research projects on rare diseases funded at all appropriate levels, including the Community level.

- Include in their plans or strategies provisions aimed at fostering research in the field of rare diseases.

- Facilitate, together with the Commission, the development of research cooperation with third countries active in research on rare diseases and more generally with regard to the exchange of information and the sharing of expertise.”

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

EUROPLAN RECOMMENDATIONS

- R3.1 Dedicated national research programs for rare diseases (basic, translational, clinical, public health and social research) are established and supported with dedicated funds, preferably for a long period.

  Research projects on rare diseases should be made identifiable and traceable within broader national research programs.

- R3.2 Specific provisions are included in the National Plans or Strategies to promote appropriate collaborations between Centres of Expertise and/or other structures of the health system and health and research authorities in order to improve knowledge on different aspects of rare diseases.

EUROPLAN RECOMMENDATIONS

- R3.3 National networks are promoted to foster research on rare diseases. Special attention is given to clinical and translational research in order to facilitate the application of new knowledge into rare disease treatment. Compilation and updating of a directory of teams carrying out research on rare diseases should be endorsed when feasible.

- R3.4 Proper initiatives are developed to foster participation in cooperative international research initiatives on rare diseases, including the EU framework programme and E-RARE. The national funding of these initiatives should be increased considerably.

- R3.5 Specific technological platforms and infrastructures for rare disease research, including clinical research, are established and supported and the creation of public-private partnership is explored.

EUROPLAN RECOMMENDATIONS

- R3.6 Multi-centre national and trans-national studies are promoted, in order to reach a critical mass of patients for clinical trials and to exploit international expertise.

- R3.7 Specific programs are launched for funding and/or recruitment of young scientists on rare diseases research projects.

- R3.8 The assessment of already existing drugs in new combinations and in new indications is supported since it may be a cost-effective way to improve treatment for patients with rare diseases.

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RELEVANT EXTRACT FROM THE

EUROPLAN INDICATORS

TO EVALUATE THE ACHIEVEMENTS OF RD INITIATIVES

EUROPLAN INDICATORS

ACTIONS	INDICATORS	TYPE	ANSWERS
Building a research programme for Rare Diseases	Existing of RD National/Regional research programmes	Process	Specific research programme for RD RD research programme included in the general research programme as a priority Not RD research programme
	RD research programme monitoring	Process	Not existing, not clearly stated Existing, clearly stated, partly implemented Existing, clearly stated and substantially implemented
	Number of RD research projects approved by year (if possible yearly starting the year before plan commencement)	Outcomes	Percentage of RD projects by the total of projects approved

EUROPLAN INDICATORS

ACTIONS	INDICATORS	TYPE	ANSWERS
Building a research programme for Rare Diseases	Clinical trials funded by public bodies	Outcomes	Yes, action implemented No actions have been taken Under discussion
	E-RARE joining	Process	Ongoing In process not considered
	Including public health and social research, in the field of rare diseases	Process	Yes No Under discussion
	Research platforms and other infrastructures are also funded by the research programme	Process	Yes No Under discussion

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EUROPLAN INDICATORS

ACTIONS	INDICATORS	TYPE	ANSWERS
Existence of national policy in support of the recruitment of young scientists / Researchers specifically for Rare Diseases	Number of young scientists recruited every year to work specifically on rare diseases	Process	Number great equal zero
Allocate funds for the RD research programme	There are specific public funds allocated for RD research	Process	Yes No Under discussion
	Funds specifically allocated for RD research actions /projects per year since the plan started	Outcomes	Million Euros allocated to RD research projects Percentage of funds allocated for RD projects by the total funds for projects

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FURTHER GUIDELINES FOR DISCUSSION

MAPPING OF EXISTING RESEARCH RESOURCES, INFRASTRUCTURES AND PROGRAMMES FOR RDs

- Evaluation of RD research resources and infrastructures across different disciplines and sources of funds, both public and private. Considering whether a combination of private and public support is feasible.
- Does a specific national RD research programme with dedicated funds exist? Is there a scope for such programme?
- What is the scope of patient-driven research?

- Further suggestion

  - Specific area: Biobanks and databases

NEEDS AND PRIORITIES FOR RESEARCH

- Assessing needs and priorities for basic, clinical and translational research, as well as priorities for social research in the field of rare diseases

FOSTERING INTEREST AND PARTICIPATION OF RESEARCHERS & PATIENTS IN RD RESEARCH PROJECTS

- How to make the link between basic and translational research and Centres of Expertise?
- Promoting interdisciplinary approaches to research
- Strengthening the exchanges among patient organisations
- Specific programmes for funding or recruiting young scientists on RD research

SUSTAINABILITY OF RESEARCH ON RD

- How to ensure, through appropriate funding mechanisms, structural and long-term sustainability of research projects and research infrastructures in the field of RDs? In particular in respect of public health and social research, as well as transversal infrastructures

EU COLLABORATION RESEARCH ON RD

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

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PROPOSALS FROM THE AUDIENCE