EURORDIS
RARE DISEASES EUROPE

RESEARCH

&

DEVELOPMENT OF

ORPHAN DRUGS,

INNOVATIVE TREATMENTS

TABLE OF CONTENT

- Relevant extracts from the **RECOMMENDATION of the Council of the European Union** on “an action in the field of rare diseases”.
- Relevant extracts from the **RECOMMENDATION of the EUCERD** - European Union Committee of Experts on Rare Diseases – **on** ‘**Core Indicators**’ for planning, implementing and monitoring national Rare Disease Plan or Strategy.

  The EUCERD brought together the 28 EU Member States plus Norway, Iceland and Switzerland, and stakeholders from patients’ organisations, academia and industry.
- Relevant extracts from the **RECOMMENDATION of EUROPLAN** – EU co-funded project aimed at developing and implementing national Rare Disease Plan or Strategy.
- Relevant extracts from **EUROPLAN Indicators**.

Relevant extracts from the RECOMMENDATION of the EU COUNCIL on an action in the field of rare diseases

(2009/C 151/02)

8 June 2009

EU COUNCIL RECOMMENDATION

"HEREBY RECOMMENDS that Member States:

6. 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.

7. 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.

EURORDIS RARE DISEASES EUROPE

EU COUNCIL RECOMMENDATION

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

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

10. 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.

EURORDIS
RARE DISEASES EUROPE

EU COUNCIL RECOMMENDATION

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

    (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 extracts from the EUCERD CORE INDICATORS for RD National Plans / Strategies

EUCERD
European Union Committee of Experts on Rare Diseases

EUCERD CORE INDICATORS

N°4. Adoption of the EU RD definition

Short Definition =
Definition as laid down in OD Regulation EC 141/2000 &
Cross Border Health Care Directive 2011/24/EU:
"no more than 5 patients per 10 000 persons"

N°12. Existence of a national policy on registries or data collection on RD

Public support for their development and sustainability

N°13. Existence of RD research programmes and/or projects in the country

Describe the status of RD research in the country

EURORDIS
RARE DISEASES EUROPE

EUCERD CORE INDICATORS

N°14. Participation in European and international research initiatives

Ex: IRDiRC or other EU / international programmes

N°15. Number of Orphan Medical Products (OMPs) with a EU marketing authorisation and available in the country (i.e. priced and reimbursed or directly supplied by the national health system)

Identify access to OMPs in your country compared to all OMPs being granted an EU marketing authorisation

N°16. Existence of a governmental system for compassionate use of medicinal products

Does a national system exist to provide medicines to RD patients prior to marketing autorisation?

EUCERD CORE INDICATORS

N° 20. Specific public funds allocated for RD research

Policy decision(s) to allocate a portion of the National research budget specifically to RD research

N°21. Public funds specifically allocated for RD research actions/projects per year since the plan started

Total amount of public funds (in EUR) allocated to RD research projects or programmes

Relevant extracts from the EUROPLAN RECOMMENDATIONS for the development of RD National Plans/ Strategies

EUROPLAN
European Project for Rare Diseases National Plans Development

EUROPLAN RECOMMENDATIONS

- R 2.8 International, national and regional registries for specific rare diseases or groups of rare diseases are promoted and supported for research and public health purposes, including those held by academic researchers.
- R 2.9 Collection and sharing of data from any valid sources, including Centres of Expertise, and their availability for public health purposes is promoted by public health authorities, in compliance with national laws.
- R 2.10 Participation of existing national registries in European /International registries is fostered.
- R 2.11 Instruments are identified for combining EU and national funding for registries.

EURORDIS RARE DISEASES EUROPE

EUROPLAN RECOMMENDATIONS

- R 3.1 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.

- R 3.2 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.

EURORDIS
RARE DISEASES EUROPE

EUROPLAN RECOMMENDATIONS

- R 3.3 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.
- R 3.4 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.
- R 3.5 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.

EURORDIS RARE DISEASES EUROPE

EUROPLAN RECOMMENDATIONS

- R 3.6 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.
- R 3.7 Specific programs are launched for funding and/or recruitment of young scientists on rare diseases research projects.
- R 3.8 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.
- R 4.5 Travelling of biological samples, radiologic images, other diagnostic materials, and e-tools for tele-expertise are promoted.

EURORDIS
RARE DISEASES EUROPE

EUROPLAN RECOMMENDATIONS

- R 5.1 The use of international global information websites and data repositories for rare diseases is promoted.
- 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.
- 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.

EUROPLAN RECOMMENDATIONS

- R 5.13 Patients' access to authorised treatment for rare disease including reimbursement status, is recorded at national and/or EU level.
- R 5.14 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.

EURORDIS RARE DISEASES EUROPE

Relevant extracts from the EUROPLAN INDICATORS for the development of RD National Plans/ Strategies

EUROPLAN
European Project for Rare Diseases National Plans Development

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)	Outcome	Percentage of RD projects by the total of projects approved

EURORDIS RARE DISEASES EUROPE

EUROPLAN INDICATORS

ACTIONS	INDICATORS	TYPE	ANSWERS
Building a research programme for Rare Diseases	Clinical trials funded by public bodies	Outcome	☐ Yes, action implemented ☐ No actions have been taken ☐ Under discussion
	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

EURORDIS RARE DISEASES EUROPE

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	Outcome	□ Million Euros allocated to RD research projects □ Percentage of funds allocated for RD projects by the total funds for projects

EUROPLAN INDICATORS

ACTIONS	INDICATORS	TYPE	ANSWERS
Develop Screening Policies	Number of diseases included in the neonatal screening programme	Outcome	Number of diseases
	Number of diseases included in the neonatal screening programme properly assessed	Outcome	Index based on the number of disease tests assessed and included in the neonatal screening programme divided by the total number of diseases included in the neonatal screening programme

EUROPLAN INDICATORS

ACTIONS	INDICATORS	TYPE	ANSWERS
Ensure the mechanism that facilitates Orphan Designated Drugs (ODD) access and reimbursement of their cost to patients after they got the market authorisation (MA) by EMA	Number of ODD MA by EMA and placed in the market in the country	Outcome	Index based on Number of ODD placed in the market by total of ODD approved by the EMA
	Time between the date of a ODD MA by EMA and its actual date of placement in the market for the country	Outcome	Average days since the date of market authorisation by EMA until the official date of placement in the market in the country
	Time from the placement in the market in the country to the positive decision for reimbursement by public funds	Outcome	Average days since the date of placement in the market until the reimbursement decision date in the country
	Number of ODD reimbursed 100%	Outcome	Number ranging 0 to 1,000

EURORDIS RARE DISEASES EUROPE

EUROPLAN INDICATORS

ACTIONS	INDICATORS	TYPE	ANSWERS
To develop mechanisms to accelerate ODD availability	Existence of a governmental program for compassionate use for Rare Diseases	Outcome	☐ Yes ☐ No ☐ In process