RESEARCH & DEVELOPMENT OF ORPHAN DRUGS, INNOVATIVE TREATMENTS
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
"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.
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.
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
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
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 authorisation?
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 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.
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.
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.
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.
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.
Relevant extracts from the EUROPLAN INDICATORS for the development of RD National Plans/Strategies
# EUROPLAN INDICATORS

<table>
<thead>
<tr>
<th>ACTIONS</th>
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<th>TYPE</th>
<th>ANSWERS</th>
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</table>
| 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 |
<p>|                                              | 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 |</p>
<table>
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| 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 |
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<tbody>
<tr>
<td>Existence of national policy in support of the recruitment of young scientists / Researchers specifically for Rare Diseases</td>
<td>Number of young scientists recruited every year to work specifically on rare diseases</td>
<td>Process</td>
<td>Number greater equal zero</td>
</tr>
<tr>
<td>Allocate funds for the RD research programme</td>
<td>There are specific public funds allocated for RD research</td>
<td>Process</td>
<td>Yes, No, Under discussion</td>
</tr>
<tr>
<td></td>
<td>Funds specifically allocated for RD research actions /projects per year since the plan started</td>
<td>Outcome</td>
<td>Million Euros allocated to RD research projects, Percentage of funds allocated for RD projects by the total funds for projects</td>
</tr>
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<tr>
<td>Develop Screening Policies</td>
<td>Number of diseases included in the neonatal screening programme</td>
<td>Outcome</td>
<td>Number of diseases</td>
</tr>
<tr>
<td></td>
<td>Number of diseases included in the neonatal screening programme properly assessed</td>
<td>Outcome</td>
<td>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</td>
</tr>
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<tr>
<td>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</td>
<td>Number of ODD MA by EMA and placed in the market in the country</td>
<td>Outcome</td>
<td>Index based on Number of ODD placed in the market by total of ODD approved by the EMA</td>
</tr>
<tr>
<td></td>
<td>Time between the date of a ODD MA by EMA and its actual date of placement in the market for the country</td>
<td>Outcome</td>
<td>Average days since the date of market authorisation by EMA until the official date of placement in the market in the country</td>
</tr>
<tr>
<td></td>
<td>Time from the placement in the market in the country to the positive decision for reimbursement by public funds</td>
<td>Outcome</td>
<td>Average days since the date of placement in the market until the reimbursement decision date in the country</td>
</tr>
<tr>
<td></td>
<td>Number of ODD reimbursed 100%</td>
<td>Outcome</td>
<td>Number ranging 0 to 1,000</td>
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<td>To develop mechanisms to accelerate ODD availability</td>
<td>Existence of a governmental program for compassionate use for Rare Diseases</td>
<td>Outcome</td>
<td>Yes</td>
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</table>