EUCERD Recommendations on Core Indicators for Rare Disease National Plans/ Strategies
The context

• Council Recommendation on an Action in the Field of RD (2009/C 151/02) (8 June 2009) encourages MS to elaborate a plan or strategy for rare diseases by 2013

• Indicators are an essential element of any such plan to ensure that national plans/strategies can be evaluated
Elaboration of the Recommendations

 First set of indicators elaborated by the EUROPLAN project (2008-2011)
 Indicators reconsidered in the scope of the EUCERD Joint Action through a broad consultation of stakeholders in 2013
 Smaller set of indicators that could be reasonably used for monitoring national activities, notably at European level, selected
 Recommendations on core indicators for national plans/strategies adopted at EUCERD’s last meeting in July 2013
Aim of EUCERD recommendations

• To be used by MS to provide information at European level to the EC on the implementation of the Council Recommendation on an Action in the field of Rare Diseases
  – Provide information on RD activities for the annual State of the Art Report
• To help MS devise appropriate indicators for their RD national plans/strategies based on these core indicators
• To serve as the basis for elaboration of indicators at national level tailored to the specific actions of plans for appropriate monitoring of measures
Structure of the Document

• Background to the recommendations
• Methodology
• Target groups
• Mission, scope and vision of the recommendations
• List of core indicators
• Definitions and associated answers
Target Groups for the Recommendations

- Policy makers in EU Member States
- European Commission

Dissemination to other stakeholders is encouraged
RECOMMENDATIONS
Categories of indicators

- Background indicators
- Content indicators
- Financial support indicators

- A detailed annex explaining the scope of each indicator and related concepts is included
Background Indicators

• Existence of regulations/laws, or equivalent official national decisions that support the establishment and development of a Rare Diseases (RD) plan
• Existence of a RD advisory committee
• Permanent and official patients’ representation in plan development, monitoring and assessment
• Adoption of the EU RD definition
Content Indicators

Centres of expertise/ European Reference Networks

• Existence of a national policy for establishing Centres of Expertise (CE) on RD

• Number of national and regional Centres of Expertise adhering to the national policy

• Participation of national or regional Centres of Expertise in European Reference Networks (ERNs)
Content Indicators

Information

• NP/NS support to the development of/participation in a comprehensive national and/or regional RD information system

• Existence of Help lines for RD

• Existence of a national policy on rare disease clinical practice guidelines development and implementation
Content Indicators

Codification and registration

• Type of classification/coding used by the health care system

• Existence of a national policy on registries or data collection on RD
Content Indicators

Research

• Existence of RD research programmes and/or projects in the country

• Participation in European and international research initiatives
Content Indicators

Orphan medicinal products

• 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)

• Existence of a governmental system for compassionate use of medicinal products
Content Indicators

Patient support

- Existence of programmes to support the integration of RD patients in their daily life
Financial Support Indicators

• Existence of a policy/decision to ensure long term sustainability of the RD plan/strategy

• Amount of public funds allocated to the RD plan/strategy

• Specific public funds allocated for RD research

• Public funds specifically allocated for RD research actions/projects per year since the plan started
Thank you for your attention!

The text of the recommendation can be found on the EUCERD website

www.eucerd.eu