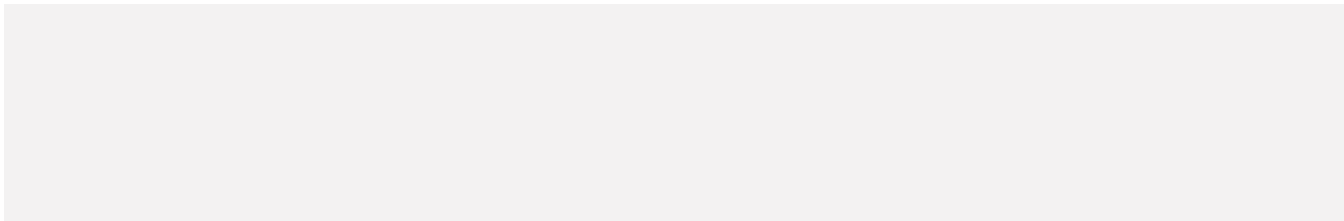




# **EUCERD Recommendations on Core Indicators for Rare Disease National Plans/ Strategies**



# 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](http://www.eucerd.eu)