The importance of European Best Practice Guidelines: RARE-Bestpractices Project and the involvement of EURORDIS

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The importance of European Best Practice Guidelines: RARE-Bestpractices Project and the involvement of EURORDIS

- **Definitions and background of the RARE-Bestpractices project**
- **Presentation of the RARE-Bestpractices project and benefits for patients**
- **EURORDIS involvement in the project**

A brief definition of best practice guidelines

- In health care, **guidelines** are **systematically developed statements** which **assist providers, patients and stakeholders to make informed decisions about appropriate health care** for specific circumstances, including clinical interventions, public health activities, or government policies. (*RARE-Bestpractices project, Glossary*)

- **Best practice guidelines** are defined as "statements that include **recommendations intended to optimize patient care that are informed by a systematic review of evidence and an assessment of the benefits and harms of alternative care options**" (*Institute of Medicine, 2011*)

- Best practice guidelines have been demonstrated to be effective means for accelerating **translation of scientific evidence into health care practice** and **reducing uncertainty in clinical practice.**

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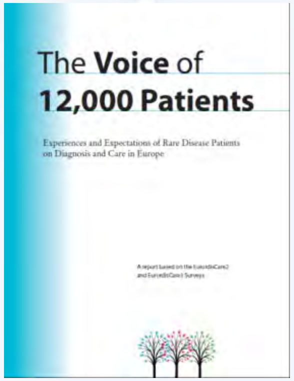
Background:
The importance of guidelines for RD patients



The **Voice** of
12,000 Patients

Experiences and Expectations of Rare Disease Patients
on Diagnosis and Care in Europe

EURORDISCARE SURVEYS: FINDINGS ON RARE DISEASE DIAGNOSIS AND CARE:

- 3 EurordisCare surveys on Experiences and Expectations of Rare Disease Patients on Diagnosis and Care in Europe and **Publication: “The Voice of 12 000 Patients”**

1. Rare disease patients experience **difficulties and delays for obtaining an accurate diagnosis.**
2. Rare diseases require **complex care** and more **coordination and communication between health professionals**.
3. Difficulties and inequalities of access to medical and/or social services result in **differences in quality of life and in life expectancies across Europe**.

➔ **Health care guidelines are a necessity for patients with rare diseases.**

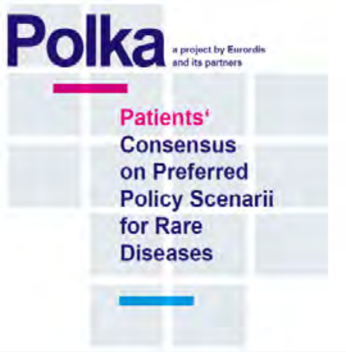
➔ These guidelines should be broad, comprehensive, including paramedical treatments, use of medical devices, physiotherapy, nutrition, surgery and complementary treatments.

Background: RD patients contribution to guidelines development

Patients organisations should be considered as research partners and as key stakeholders in the drafting of guidelines and policies:

- **Identify the existing Clinical Practice Guidelines** concerning their disease
- Take part in the **elaboration, dissemination and follow-up of guidelines**
- Contribute to the **development of best practices guidelines regarding the day-to-day care management**
- Contribute to **guidelines' dissemination via patients' associations.**

Background:
Recognising the need for patient involvement in guideline development


Polka a project by Eurordis and its partners

Patients' Consensus on Preferred Policy Scenarii for Rare Diseases

- **EURORDIS, Polka project: Patients' Consensus on Preferred Policy Scenarii for Rare Diseases** (2008-2011)

Two major outcomes:

➔ Declaration of Common Principles on Centres of Expertise and European Reference Networks for Rare Diseases, EURORDIS

> *13. **Centres of Expertise and European Reference Networks shall provide guidelines on the most appropriate care management for patients**, closely integrating both medical and social aspects. They should **involve patients and give them an active role** as **recognised partners** at all stages.*

➔ Good practices Charter of relations between Centres of Expertise (CoE) and Patient Organisations

> *Centres of Expertise should consult patients regarding drafts, involve them in working groups and disseminate information to them on Standards of Diagnosis and Care.*

EURORDIS Rare Diseases Europe

Background: European policy

- **Directive 2011/24/EU of the European Parliament and of the Council on the application of patients' rights in cross-border healthcare:**

➔ **Article 12** : encourages the development of **European Reference Networks (ERNs)** in the area of rare diseases which **should** ***"offer a high level of expertise and should have the capacity to produce good practice guidelines*** *(…)".*

- **EUCERD Recommendations on Rare Diseases ERNs, January 2013:**

Analysis of the previous and current pilot European Reference Networks (ERNs) funded by DG Sanco or by DG Research during the EU programme 2008-2013
➔ the most valuable services developed by these networks include, among others:

Common production of guidelines/best standards of diagnosis and care *and information packages, training tools and training sessions covering both the medical and the social dimension of care.*

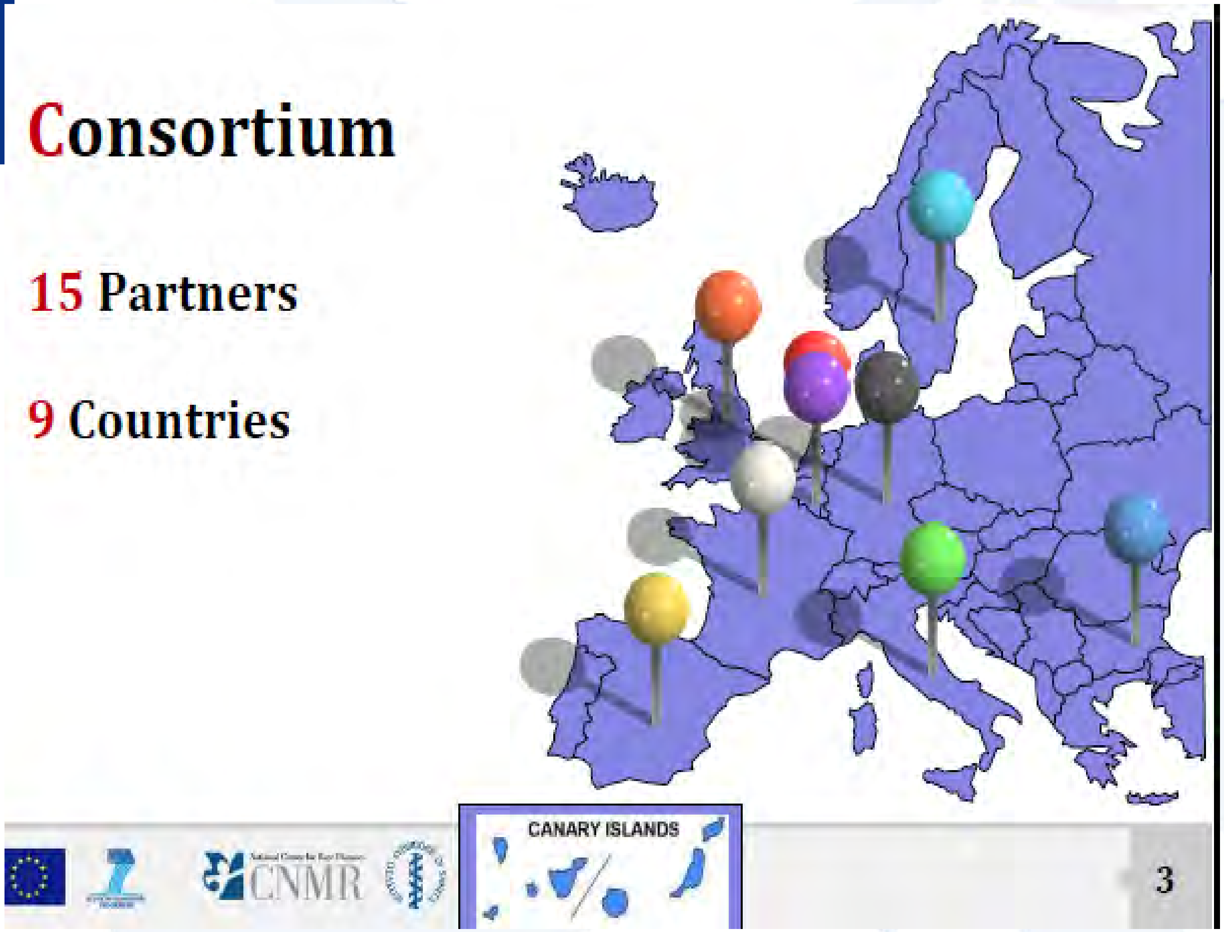
➔ **Best practices guidelines should be developped and disseminated across EU and non-EU countries** to support a consistent quality of healthcare services for RD patients in EU Member states and implement the EU directive on cross-border healthcare.

RARE-Bestpractices project

- A 4 years project : 2013-2016
- A European project Funded by DG Research under FP7-HEALTH-2012 INNOVATION-1
- Coordinated by the National Centre for Rare Diseases-Italian National Institute of Health.
- http://www.rarebestpractices.eu

- **A platform for sharing best practices for the management of rare diseases**
- This project will develop a sustainable platform for sharing best practices for the management of rare diseases, supporting the collection of standardized and validated data, the efficient exchange of knowledge and of reliable information on rare diseases.
- **Expected impact: An improvement of health outcomes and quality of life for RD patients,** by reducing inequality in RD care and by ensuring equity of access to better diagnostics and therapeutics at EU level.

RARE-Bestpractices project: Partners



Consortium

15 Partners

9 Countries

Partners

Click on the institution's name for names and a profile of collaborators

- Istituto Superiore di Sanità - National Centre for Rare Diseases
- Jamarau
- Karolinska Institutet
- Healthcare Improvement Scotland
- London School of Economics and Political Science
- National Research Council
- EURORDIS, European Organisation for Rare Diseases
- Associazione per la Ricerca sull'Efficacia dell'Assistenza Sanitaria Centro Cochrane Italiano
- Universitaetsklinikum Freiburg
- Bulgarian Association for Promotion of Education and Science
- Fundación Canaria de Investigación y Salud
- Universiteit Maastricht - Institute for Public Health Genomics (IPHG)
- Newcastle University Upon Tyne
- The European Academy of Paediatrics
- Instituto de Salud Carlos III

RARE-Bestpractices project

Rare
Best
Practices

Project aims:

- The **collection, evaluation and dissemination** of existing best practices;
- An **agreed methodology** suitable to develop and update best practice guidelines;
- A **forum for exchanging information**, sharing lessons learnt and facilitating collaborations.

Objectives :

1. To capitalize on existing best practices documents
2. To systematically identify gaps in scientific knowledge as well as related research needs, and recommend relevant research initiatives
3. To develop an international, innovative, efficient framework to build consensus on the methodology suitable for the development and implementation of best practice guidelines for rare diseases
4. To provide training activities targeted to different stakeholders to share expertise and knowledge
5. To foster synergistic collaborations among agencies, institutions, networks and organizations experienced in best practices guideline development.

RARE-Bestpractices project: Expected benefits for patients

- Best practices guidelines have been demonstrated to be **effective means for accelerating translation of scientific evidence** and **reducing uncertainty in clinical practice.**

➔ The project aims at taking better advantage of scientific progress in biomedical research in ways **to translate research results into tangible benefits for RD patients in a timely manner.**

- The project aims at promoting and supporting **a consistent level of healthcare services to patients in EU member states** (thus implementing the EU Directive on the application of patients rights in cross border healthcare).

➔ The ultimate **impact of the project should be a significant improvement of health outcomes and quality of life for RD patients**, by reducing inequality in RD care and by ensuring more equity of access to better diagnostics and therapeutics at EU level.

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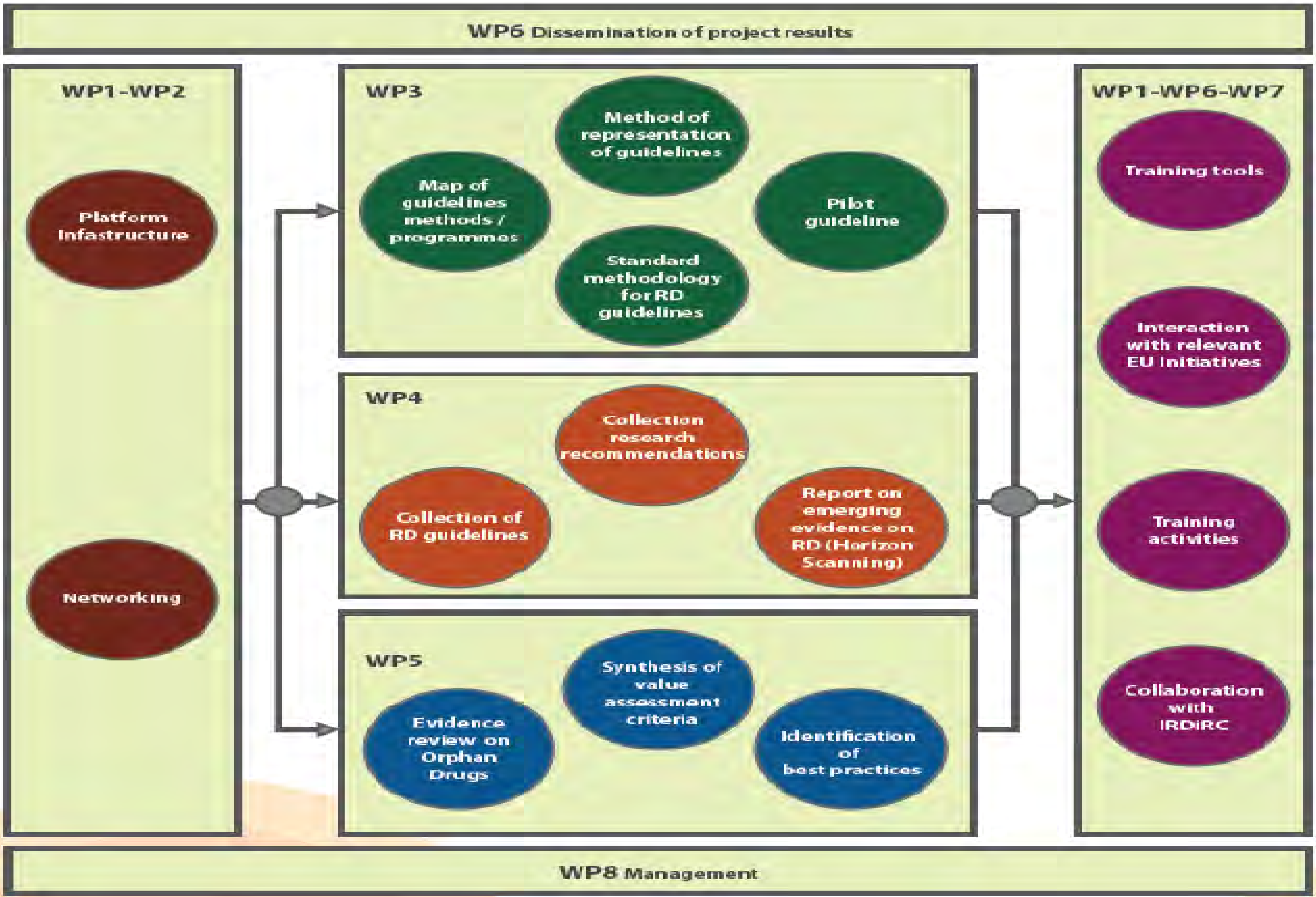
RARE-Bestpractices project: Work Packages



WP6 Dissemination of project results

WP1-WP2

Platform Infrastructure

Networking

WP3

Method of representation of guidelines

Map of guidelines methods / programmes

Pilot guideline

Standard methodology for RD guidelines

WP4

Collection research recommendations

Collection of RD guidelines

Report on emerging evidence on RD (Horizon Scanning)

WP5

Synthesis of value assessment criteria

Evidence review on Orphan Drugs

Identification of best practices

WP1-WP6-WP7

Training tools

Interaction with relevant EU Initiatives

Training activities

Collaboration with IRDiRC

WP8 Management

RARE-Bestpractices project: EURORDIS involvement

- **EURORDIS will act as a transversal partner** in the different work packages, ensuring the involvement and the capacity-building of patients organisations across Europe regarding the project.

- **EURORDIS is especially involved in the following work packages :**
  - **WP 1 -** Scientific coordination, networking
  - **WP 3 -** Agree upon methodology for production of guidelines on clinical management of RD
  - **WP 6 –** Dissemination.

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RARE-Bestpractices project: Involvement of patient organisations

- EURORDIS is involved in the development of **training initiatives**, to promote the development and use of high quality guidelines and **to build capacities among patient representatives.**
- Building on training initiatives such as **EURORDIS Summer School**.
- Establishing **synergies with other actions in the field of rare diseases** such as EUROPLAN, European Commission Expert Group on Rare Diseases, etc.
- **Disseminating information and organising special sessions during EURORDIS conferences and events** (EURORDIS Membership Meeting, Council of European Federations, etc.)
- Contributing to the **organisation of the final symposium.**

RARE-Bestpractices project: Involvement of patient organisations

- Involvement in the common work to agree on **methodological quality standards for the development of guidelines** on clinical management of rare diseases.

- EURORDIS is task leader for the development of a **patient version of the pilot best practice guideline** for a specific rare condition, implementing the project methodological quality standards.

  This patient version will provide a summary of recommendations made for health professionals, in a language **adapted for patients and their families**.

- EURORDIS will ensure the **involvement of patients and their families in the elaboration process,** relying on its network of patient organisations across Europe.

RARE-Bestpractices project: Involvement of patient organisations

WP4

Collection of RD guidelines

Patient organisations actively contribute to guidelines collection:

- The RARE-Bestpractices platform will include a **database** to help identify guidelines for rare diseases.
- Once the database has been launched, patient organisations will be able **to publish their guidelines directly to the database** and eventually, the database will contain guidelines on all rare disease topics.

- As a first step in this direction, **a sample of existing rare disease guidelines is being collected for inclusion** in the database.

- Patient organisations members of the Council of European Federations **have been invited to inform this activity**
  ➔ survey asking them to suggest specific named disease or syndrome to include in the sample and to provide project partners with some information about the existing guidelines for this disease.

RARE-Bestpractices project: Involvement of patient organisations

A patient working group on best practice guidelines:

- EURORDIS is currently recruiting patient volunteers willing to be involved in a **working group on best practice guidelines** and **share their expertise**.
- The creation of this working group will allow us to collect and synthetise the **expectations from a diverse group of patient representatives** and to get their **advice on project activities**.
- This will also **serve patient organisations needs** in developing best practice guidelines.

➔ **Get involved** to contribute and to benefit from the knowledge generated by the project!

THANK YOU!

If you would like to know more and to get involved:

Website: http://www.rarebestpractices.eu/

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