



RARE-Bestpractices project

A platform for sharing best practices
for the management of rare diseases



Presentation and update by Juliette Senecat

RARE-Bestpractices project



*Emilia - Achondroplasia
(Photo contest winner 2011)*

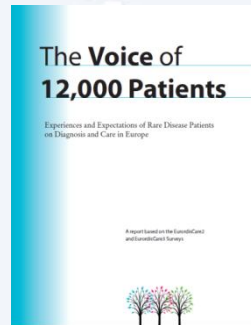
- **History and background of the RARE-Bestpractices project**
- **Presentation of the RARE-Bestpractices project**
- **EURORDIS involvement**
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- **EURORDIS Working group on best clinical practices: Get involved!**
- **Discussion**

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.**

Project background and history (I)

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 of the book “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 necessary for patients with rare diseases.**
- ➔ These guidelines should be broad, comprehensive, including paramedical treatments, use of medical devices, physiotherapy, nutrition, surgery and complementary treatments.

Project background and history (II)

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

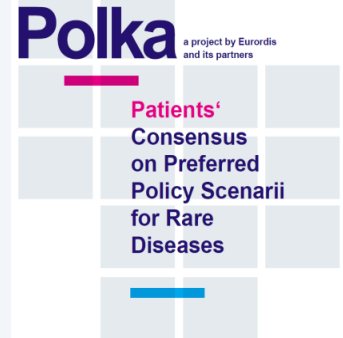
Two major outcomes:

➔ 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 in drafts, involve them in working group and disseminate information to them on Standards of Diagnosis and Care.



Project background and history (III)

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:**



The 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, shows that 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 developed 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 Istituto Superiore di Sanita – ISS, Rome.

- 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

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 Diseases and Orphan Drugs

An International Journal of Public Health

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Leaflet

Concise information about project objectives and participants

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RARE-Bestpractices project



Project aims:

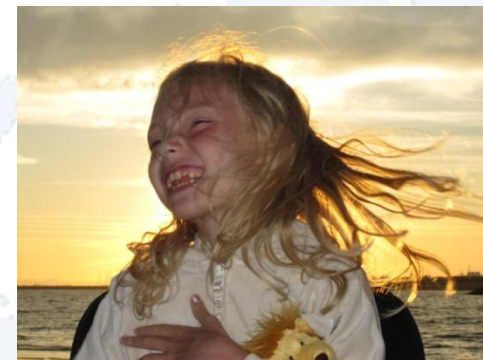
- The collection, evaluation and dissemination of existing best practices;
- An agreed methodology suitable to develop and update best practice guidelines;
- A forum for exchanging information, sharing lessons learnt and facilitating collaborations.

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: 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 equity of access to better diagnostics and therapeutics at EU level.

Rare diseases 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

RARE-Bestpractices: 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.

EURORDIS involvement: WP1

Scientific coordination and networking

EURORDIS will be especially involved in **Task 1.2 : Managing training initiatives.**

OBJECTIVE: to promote the development of high quality BP guidelines and their use across Europe.

- Training will be targeted to the anticipated needs of all relevant stakeholders involved in RD (primarily best practice guidelines developers of EU organizations and of European Reference Networks but also **patient representatives**).
- **EURORDIS will build on existing training initiatives :**
 - EURORDIS Annual Summer School for patients advocates
 - Involvement in the EUPATI project - European Patients' Academy on Therapeutic Innovation

EURORDIS involvement: WP3

Agree upon methodology for production of guidelines on clinical management of rare diseases

- EURORDIS will be task leader for [the Patient version of the pilot best practices guideline](#):
 - First, a pilot Best Practice Guideline for health care professionals will be developed to implement and test the methodological quality standards for developing guidelines for rare diseases.
 - Then, EURORDIS will work on a patient version as a complement to this pilot best practice guideline.
 - 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 RD Patients Organisations across Europe

EURORDIS Involvement: WP6

Dissemination

- EURORDIS will build capacities of RD Patient representatives on the importance, use and benefits of best practices guidelines, through dissemination of information and education of patient advocates.
- EURORDIS will help maximize the project's impact by ensuring acceptance by patient community.

Specific tasks include:

- **Task 6.2** - Organise special sessions during EURORDIS' existing conferences and events
- **Task 6.3** - Establish synergies with other actions in the field of Rare diseases such as EUROPLAN, EUCERD Joint Action, ECRIN, EPIRARE, etc.
- **Task 6.6** - Involvement in the organisation of the Final International Symposium

Update and next steps

- Three **project workshops** have been organised since January 2013:
 - Kick-off meeting, Rome, February 2013
 - Second project meeting, London, May 2013
 - Workshop on the methodology of guidelines development, Freiburg, October 2013.
- Project partners are now agreeing on a **methodology** to elaborate best practice guidelines for rare diseases (report to be published following Freiburg workshop)
- A **survey** has been sent to health authorities across Europe to ascertain existing national or local programmes and initiatives dedicated to Best Practice Guidelines on Rare Diseases in the European Member States, and methods in use to develop them.
- **Communications:** Project website; EURORDIS eNews published.
- 1st project newsletter will be released at the end of 2013.
- EURORDIS is now in the process of creating a **patient working group** on best practices in order to involve patient organisations more directly in the project.



EURORDIS working group on best practices

EURORDIS is creating a working group on best practices:

- An informal group (terms of reference to be developed).
- CEF members would have a highly valuable input.
- The creation of this working group would allow us to collect and synthesise the expectations from a diverse group of patient representatives and to get their advice on project activities.
- This would serve patients organisations needs in developing best practice guidelines.
- Your organisation would have the opportunity to benefit from the knowledge generated by the project.



Get involved!

We are going to contact you very soon with more information.



THANK YOU!

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

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

Contact: Juliette SENECAAT, Health Research Projects Manager
Juliette.senecat@eurordis.org

Many thanks to Monica ENSINI for starting the project
and to Anna KOLE for her contribution.



Points for discussion

- **What is the situation of health care guidelines regarding your disease / group of diseases?**
- **Could you tell us about your experiences of involvement in the development of guidelines?**
- **What do you expect from the RARE-Bestpractices project, and how would you like to be involved?**