European Reference Networks

2nd European Reference Networks Conference

8-9 October Lisbon, Portugal

A report by: G Porto & F Courtois

(invited by EURORDIS)

EFAPH Fe

EURORDIS
Rare Diseases Europe

summary

I – INTRODUCTION

- What does a ERN mean?

  What will we (patients) gain by belonging to na ENR?

- Why belong to EURORDIS?

  Is HH a rare disease?

- Important steps so far (since 2011)

II – A report of the LISBON MEETING, 2015

III – WHAT TO DO NEXT?

Why belong to na ERN?

The European Commission (EC) and EU Member States are working to establish European Reference Networks (ERNs) to **link existing highly specialised healthcare providers across the European Union.**

As expertise in rare or low prevalence complex diseases or conditions is scarce and dispersed, ERNs will help facilitate access to diagnosis and treatment by **centralising knowledge and experience, medical research and training, and resources in the area of rare or low prevalence complex diseases or conditions.**

The premise for establishing ERNs is that expertise should be brought closer to the patients' home.

Every patient in Europe with a rare disease should find its "home" in the network

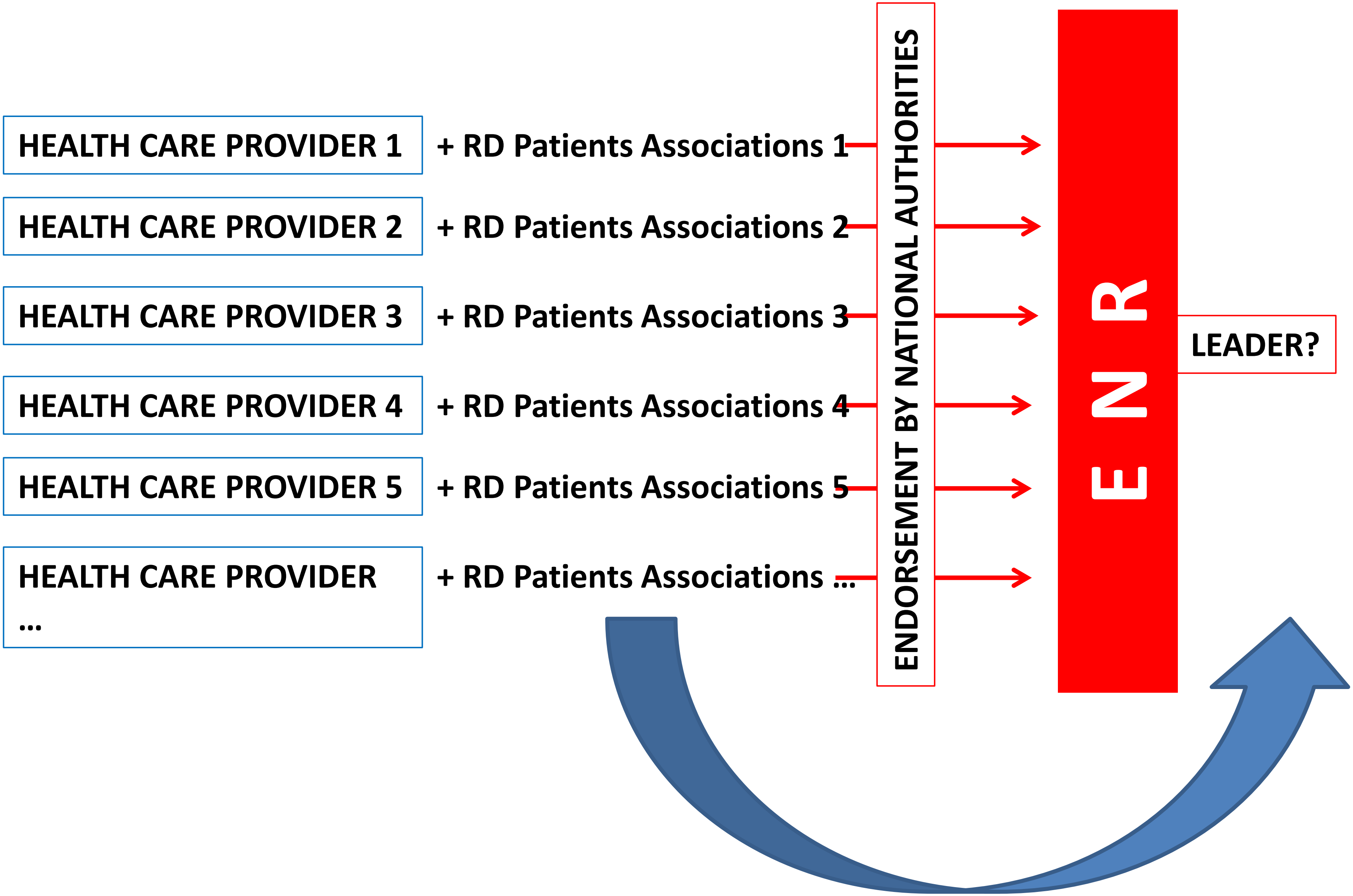
In more practical terms…

- To find a “trademark” for **good practices**

- To “equalize” practices through coordination of **patient care within and across Networks**

- To “have a voice” through the **patient and family involvement** in all processes (the role of **EURORDIS**)

HOW IS AN ERN CONSTRUCTED?



HEALTH CARE PROVIDER 1 + RD Patients Associations 1

HEALTH CARE PROVIDER 2 + RD Patients Associations 2

HEALTH CARE PROVIDER 3 + RD Patients Associations 3

HEALTH CARE PROVIDER 4 + RD Patients Associations 4

HEALTH CARE PROVIDER 5 + RD Patients Associations 5

HEALTH CARE PROVIDER … + RD Patients Associations …

ENDORSEMENT BY NATIONAL AUTHORITIES

ERN

LEADER?

Is HH a Rare Disease?

1- low prevalence?
(Rare forms of HH; low penetrance of HFE-HH)

2- The need for highly specialized Centers?
(Diagnostic tools, research)

3- Low awareness and/or knowledge
(essential for prevention)

historical background

A Lunch debate at the EU Parliament

Tuesday, September 20, 2011, (12: 30 – 14: 30)

How do we fill the information gap in Europe ?

Reccommendation:

Efforts must be put on looking for opportunities in the frame of Rare Diseases

EFAPH officially joins EURORDIS as associate member in 2012

Highlights:

2012 August 31, EFAPH AGM , Rennes: Odile Kremp **presents the ENR** project
EFAPH participates in the public consultation on ERNs in the frame of the Crossborders Directive

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2014 June 14, Networks awareness conference in Brussels: **Official launch by DG SANCO of the ERN programme** (establishes the criteria and conditions for a network, and criteria for membership) – announced by O Loreal at the EFAPH AGM, Verona

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2015	September, the **Assessment Manual** is published F Courtois participates in the EURORDIS AGM , Madrid – announced an **addendum to EUCERD recommendations** and the next Lisbon Meeting

This Addendum accompanies the *EUCERD Recommendations on Rare Disease European Reference Networks*, adopted on 31 January 2013, and complements the content of the Recommendations in the following areas:

. The **grouping** of rare diseases in Thematic Networks;

. The necessity of **Patient Involvement** in RD ERNs.

2015 October 8-9 - EFAPH is invited to participate in the EURORDIS meeting in Lisbon. The main objectives of the meeting were **to acknowledge and present the work done** so far on the ERN process, and point to future actions.

THE EURORDIS LISBON MEETING

The Meeting structure:

The Roundtables:

1. The framework for the establishment of ENRs (implementation, manual for technical assessment and approval by the Member States (MS) Board)
See at: http://ec.europa.eu/health/ern
2. Strategic value for the MS: France, Portugal and Lithuania
3. Network organizational challenges and experiences: issues, solutions and lessons learned *(from the patients 'perspective: "supporting clinical networks is the way"; addressing needs)*
4. ERNs and better health outcomes: good clinical practices
(the importance of "measuring" the outcomes)
5. Network challenges: eHealth and IT solutions
(high tech solutions to "link" existing databases)

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The Meeting structure:

The Workshops:

1. How to prepare a successful proposal to build an ENR?

2. How to develop, use and appraise clinical decision making tools?

3. Clinical Research in a Network environment

4. Rare Diseases Networks: lessons learned, grouping diseases and patient involvement

Important Message: Why aren't we ready to apply for an ERN in Rare Diseases?

The 2 critical issues:

Obligatory grouping of diseases: it is not possible to apply for any single disorder (hemochromatosis) or simple condition (iron overload) *(we have to find our group-this question will be posed by EURORDIS)*

At least 10 recognized centers (health care providers from at least 8 countries) **endorsed** and **sustained** by the respective member states *(how many of us fulfill this criterion?)*

The proposed broad grouping:

- Rare immunological and auto-inflammatory diseases
- Rare bone diseases
- Rare cancers and tumours
- Rare cardiac diseases
- Rare connective tissue and musculoskeletal diseases
- Rare malformations and developmental anomalies and rare intellectual disabilities
- Rare endocrine diseases
- Rare eye diseases
- Rare gastrointestinal diseases
- Rare gynaecological and obstetric diseases
- Rare haematological diseases
- Rare craniofacial anomalies and ENT (ear, nose and throat) disorders
- Rare hepatic diseases
- Rare hereditary metabolic disorders
- Rare multi-systemic vascular diseases
- Rare neurological diseases
- Rare neuromuscular diseases
- Rare pulmonary diseases
- Rare renal diseases
- Rare skin disorders
- Rare urogenital diseases

The **Advantages** of integrating the group of hematological disorders is**:**

1) IRON METABOLISM is a subject of Hematology.

2) Very easy to find **expert groups in basic & clinical aspects of iron metabolism** with whom we meet regularly and may establish collaborative work.

3) An already established regular communication between EFAPH and **other patients´organizations** in the field of hematological disorders, in the frame of the **EHA advocacy group**.

We have been expressly invited by EHA to participate in their initiatives at the Lisbon Meeting to support a group

THE EURORDIS LISBON MEETING

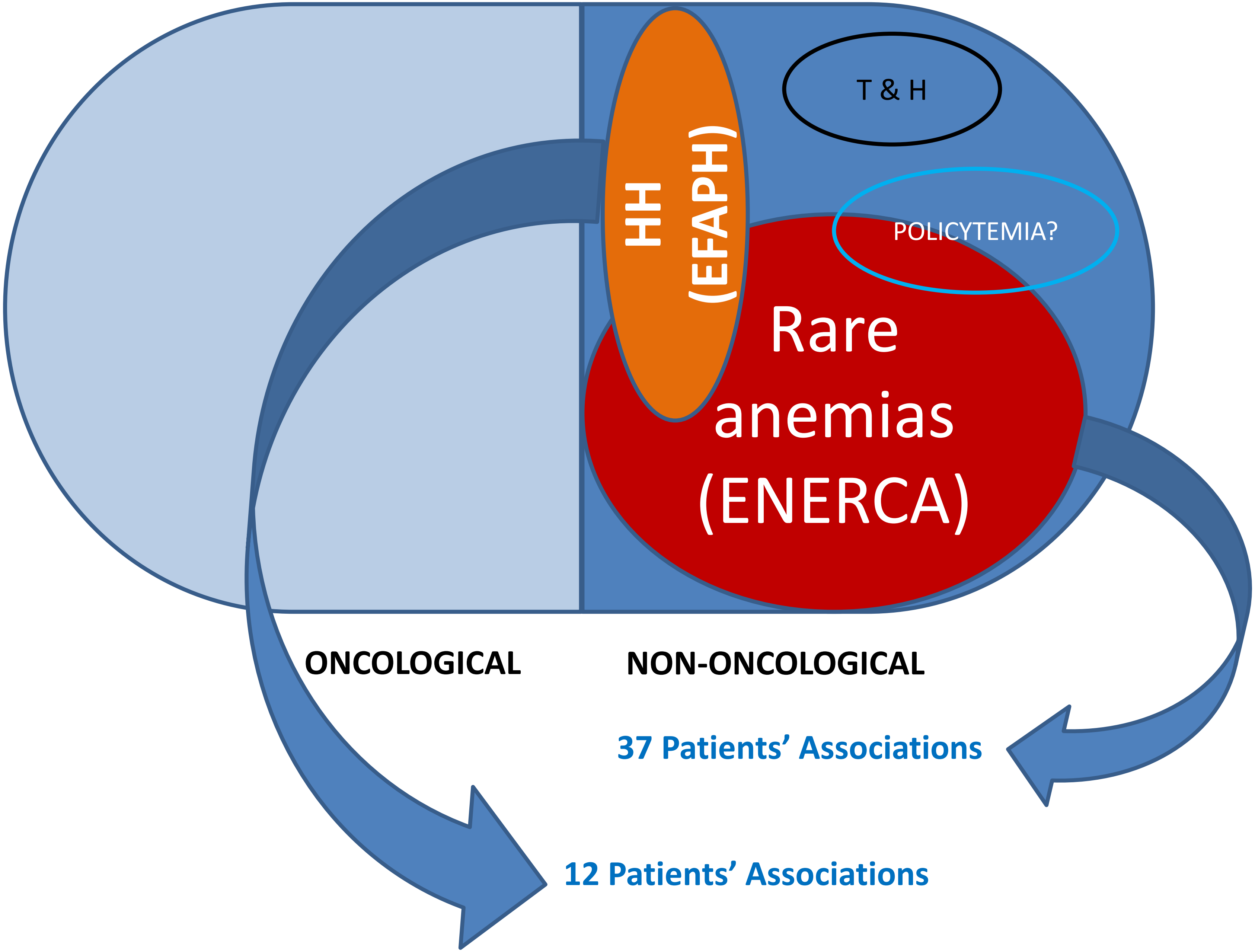
The Informal Meetings (fostering “group formation”)

The EHA advocacy group facilitated a lunch meeting with the “hematology people” to explore the possibility to foster the creation of a ERN

Participants:
From EHA: Tom de Wit & Jan Geissler
From EFAPH: Graça Porto & Françoise Courtois
From ENERCA: Vives Corrons & Maria Mañu Pereira
Independent Groups: Nika Cappelini, António Piga

Discussions addressed: 1) the importance of splitting the non-oncological disorders 2) the role of EHA as facilitator 3) the position of ENERCA as potential leader

HEMATOLOGICAL DISORDERS



HH (EFAPH)

T & H

POLICYTEMIA?

Rare anemias (ENERCA)

ONCOLOGICAL

NON-ONCOLOGICAL

37 Patients' Associations

12 Patients' Associations

WHAT TO DO NEXT?

Do we agree on the following points?

1. **What** group do we feel to belong (the EURORDIS question)

Rare Hematological Disorders

2. **Why** integrate this group? **Can we "loose our soul"**?

the natural grouping by thematic proximity (red blood cell disorders and iron metabolism)

3. **Who** will we accept as leader?

Fundamental experience with the "burden" of european burocracy

The Network must have one of the Healthcare Provider Applicants act as the **Coordinating Member**. The Coordinating Member should assign a ***Coordinator*** **(or designate**) to fulfil the following roles and responsibilities on behalf of the Network.

Accept to join the initiative of the ENERCA group as Coordinator of the ERN?

5. **How** can we work in coordination with the other groups' initiatives?

Maintain close contacts with ENERCA & EHA to follow-up actions

WHAT TO DO NEXT?

Can we all start moving?

1. Update and extend the **mapping** of candidate centers (an effort already started by O Loreal), with a focus on questions approaching the **practical self-assessment criteria and principles**

2. Each center wishing to belong to the ERN has to approach the **national authorities** to evaluate possibilities of national endorsement

3. **Patients' Associations** have to participate in the process

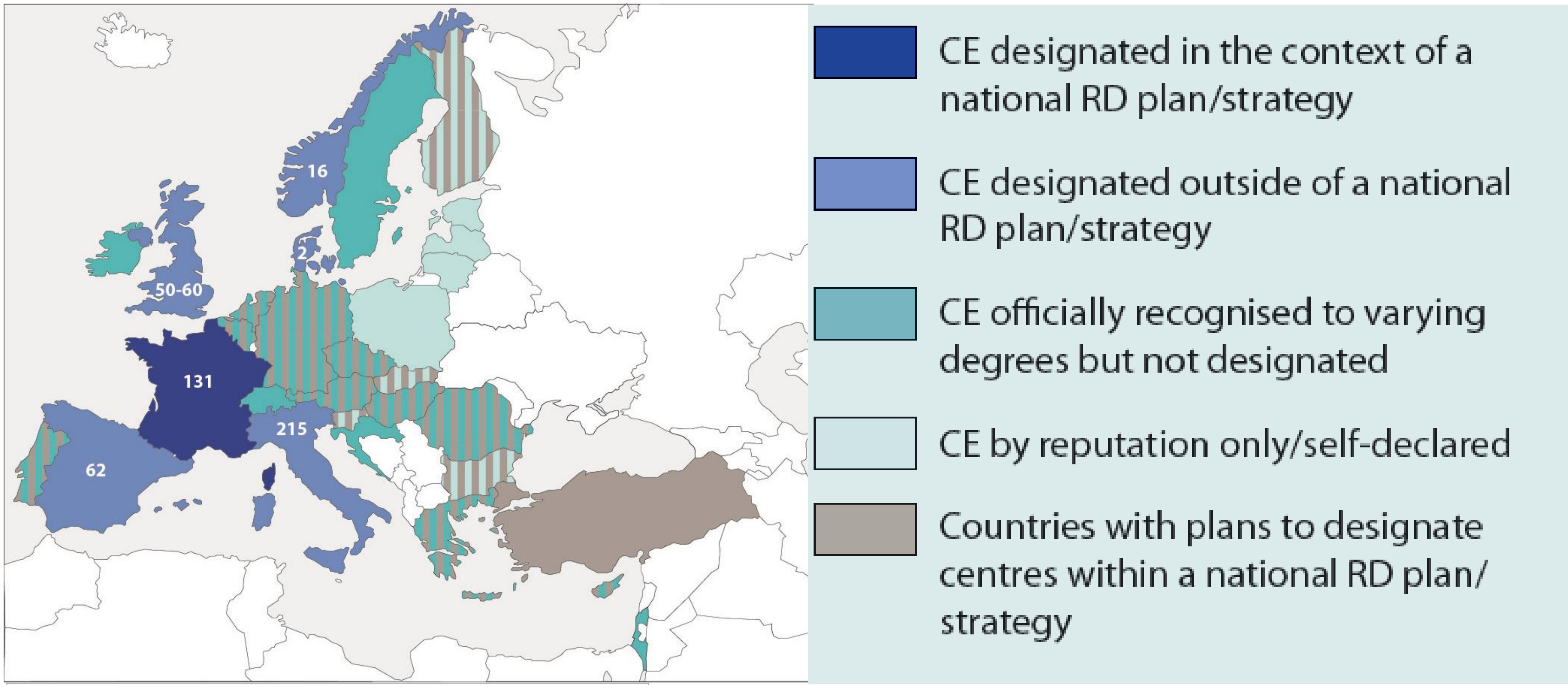
HOW TO PERFORM THE SELF-ASSESSMENT EXERCISE?

A preliminary self-assessment exercise should be done by all applicants to evaluate the compliance with the criteria established in the assessment manual (http://ec.europa.eu/health/ern)

A **coordinator** will facilitate and promote the exercise (*Domenico Girelli*)

(update the questionnaire)

HOW TO FIND OUR NATIONAL REFERENCE CENTERS FOR RD?



WHAT TO DO NEXT?

HOW IS EACH COUNTRY RECOGNISING THE RCs TO ENDORSE TO AN ERN?

Informal models:

- **Denmark, Sweden, Finland, Hungary, Slovenia, Greece, Romenia**

Usually a national health council defines and approves a set of criteria to recognize a RC with "specialized functions or competences in diagnosis and treatment". They are **generally the University Hospitals or other Centers which may have gained international recognition** trough research and publications.

Formal models with decentralized implementation (bottom-up model)

- **Germany, Belgium, France, UK**

Spontaneous applications, according to defined criteria, are **validated by a national committee for a given period of time (2-5 years).** Instruments to assure quality may include certification and accreditation.

Formal models with centralized implementation (top-down)

- **Spain, Italy, Portugal**

A committee is created in the frame of the national health council which **defines the needs, strategic locations and the priority** of diseases to be included in the ERNs

WHAT TO DO NEXT?

HOW CAN THE NATIONAL PATIENTS' ASSOCIATIONS PARTICIPATE?...

... ask yourself the following questions:

1. Is **my Country** **doing some effort** to integrate "Centers for Disorders of Iron Metabolism" in an ERN ?

2. How is **my Association** **contributing** to that effort?

(ELLABORATE A COMMON LETTER???)

CONCLUSIONS

1. An action for **EFAPH** (work on the self-assessment process)

2. An action role for each **Health Care Provider** (lo look for national endorsement)

3. An action for **Patients´s Associations** (to contact national authorities)

WHAT TO DO NEXT?

THANKS!

Basic questions for a Self-Assessment:

The Applicant is expected to define and justify the scope and thematic groups of rare or low prevalence complex disease(s) or condition(s) covered by the Network (ERN). The Applicant is also responsible for **determining and describing the characteristics required by all the Healthcare Provider Applicants for the specific condition(s)** or disease(s) covered by the Network based on epidemiological data, recognised sources and/or expert consensus, including:

- The healthcare **services to be provided** for each of the patient groups served;
- Maintenance of **competency and expertise**;
- **Qualifications** of the healthcare professionals;
- Composition of the **multidisciplinary team**;
- **Specialised resources** needed to provide quality patient care (facilities, equipment, and diagnostic services); and
- **Best practices** to be followed.

The term Applicant refers to both the Network (ERN) and the Healthcare Provider Applicants requesting to form or join an ERN.

IMPORTANT NOTE : Patients participation