RARE-Bestpractices Conference

24 November 2016, Istituto Superiore di Sanità, Rome, Italy
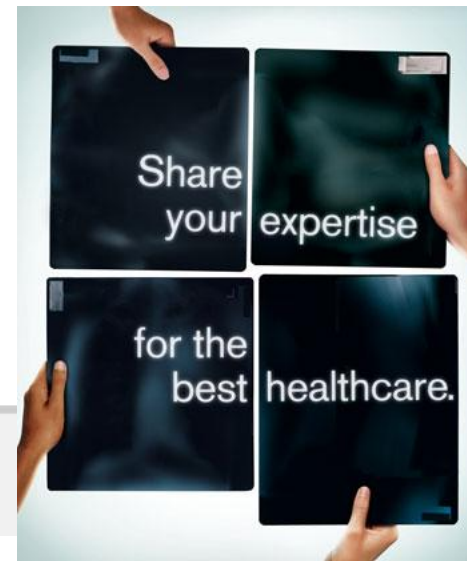
RARE-Bestpractices and European Reference Networks

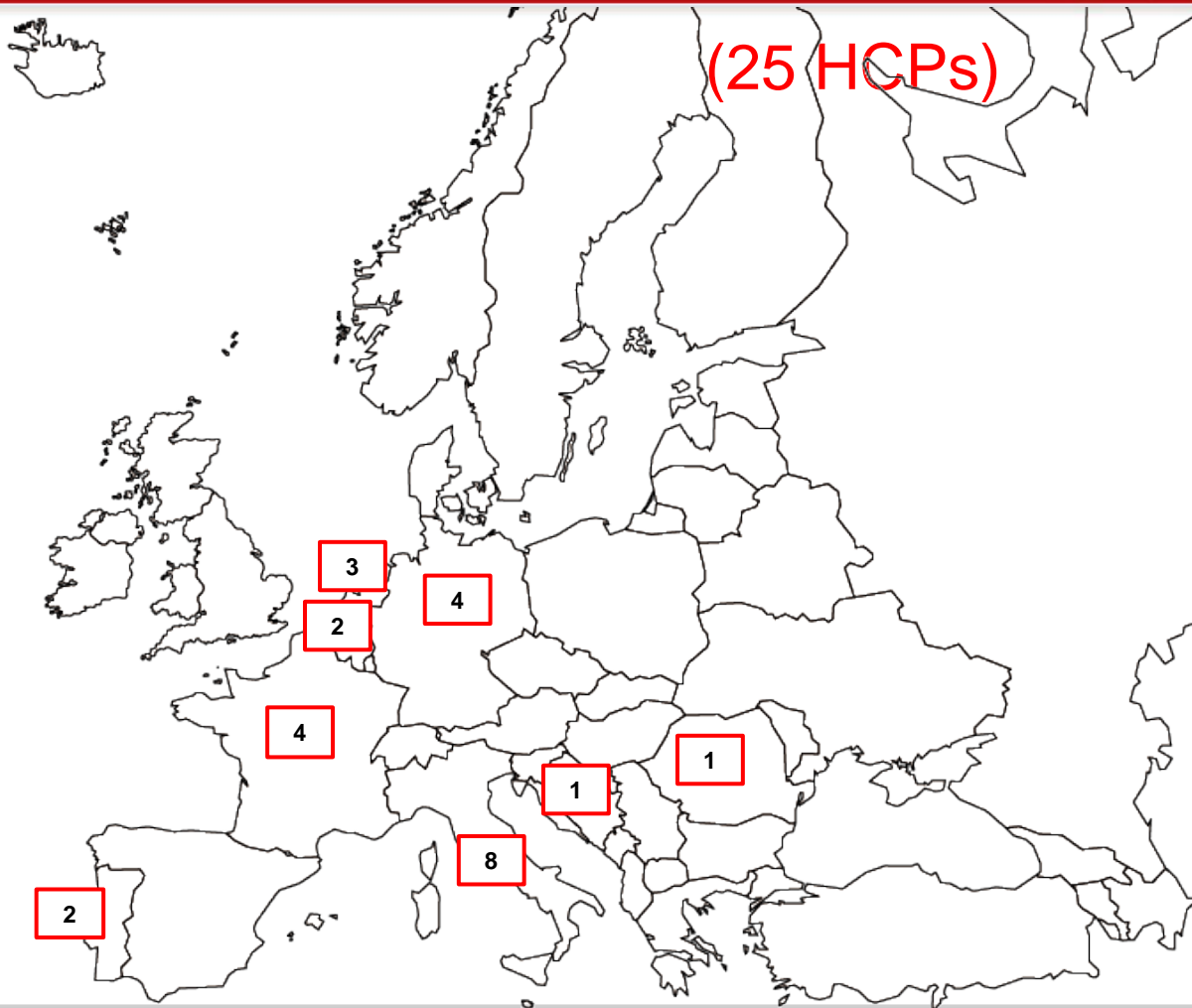*Marta Mosca, Rosaria Talarico*
U.O. Reumatologia, Università di Pisa

"…Imagine if the best specialists from across Europe could join their efforts to tackle complex or rare medical conditions that require highly specialised healthcare and a concentration of knowledge and resources."

(Terol E., 2ⁿᵈ Conference on European Reference Networks. Lisbon 8-9 October)

European Reference Networks

Share your expertise for the best healthcare.

ReCONNET

(25 HCPs)

- Belgium 2
- France 4
- Germany 4
- Italy 8
- Portugal 2
- Netherlands 3
- Romania 1
- Slovania 1

Map markers:
- 3
- 4
- 2
- 4
- 1
- 1
- 8
- 2

National Centre for Rare Diseases
CNMR

Member of
EURORDIS
Rare Diseases Europe

ReCONNET Thematic Groups

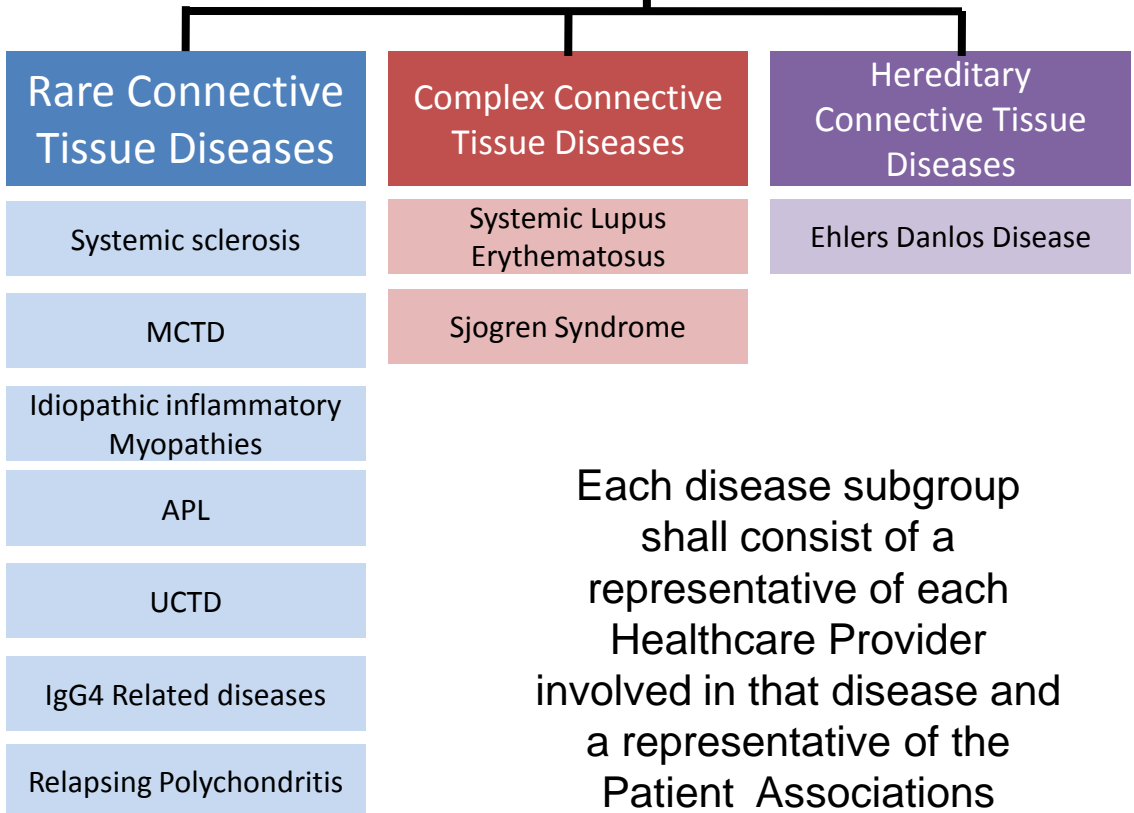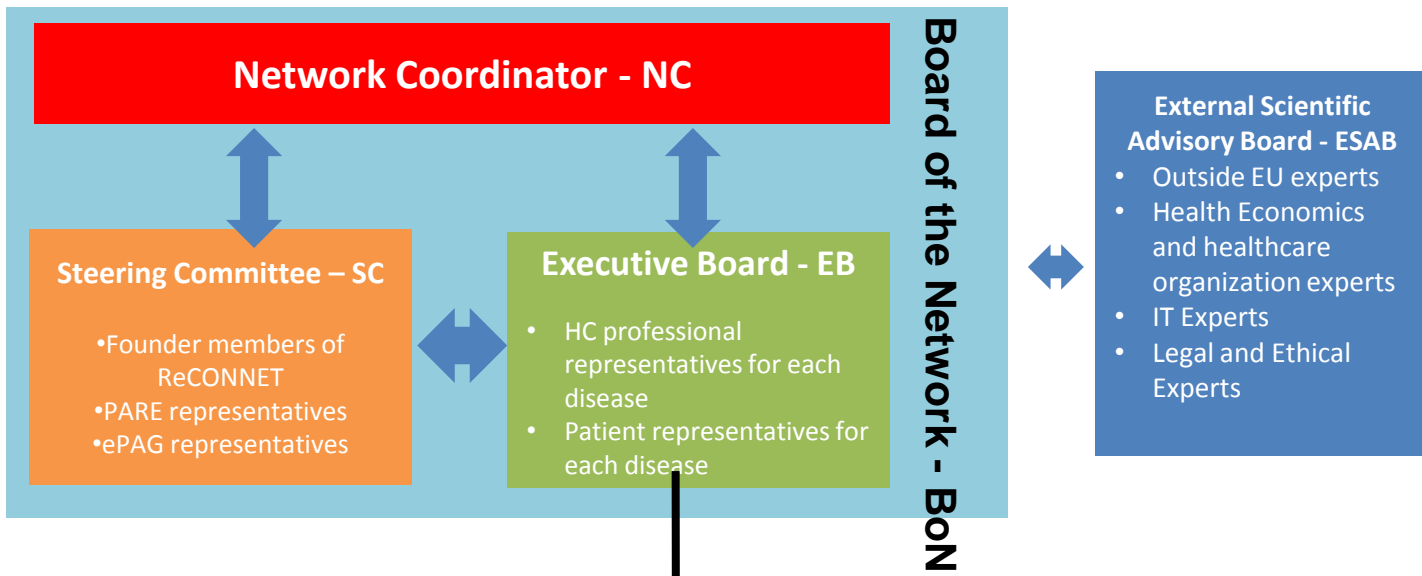| Disease |
|---|
| Systemic sclerosis |
| Mixed connective tissue diseases |
| Idiopathic inflammatory myopathies |
| Undifferentiated connective tissue diseases |
| Anti-phospholipid syndrome |
| Systemic lupus erythematosus |
| Sjogrens syndrome |
| Polychondritis |
| Ig4 related diseases |
| Ehlers Danlos |

Development of a European Network of centres of Excellence in rare and complex hereditary and autoimmune connective and musculoskeletal diseases aimed at:

- Increasing and homogenizing quality of care across EU borders and developing strategies for cross border movement of patients.

- Empowering patients in the management of their disease.

- Optimizing available resources and deliver cost effective care.

- Increasing knowledge on rare and complex conditions.

- Facilitating epidemiological, clinical, translational research.

- Definition of existing guidelines and recommendations; assess adherence and implementation of their use in clinical practice.

- Collection of clinical cases, adverse events, drugs side effects and report to the scientific community.

- Development of recommendations/guidelines.

- Definition of pathways of care, multidisciplinary teams.

- Case discussions among centres, data sharing.

- Definition of legal and organizational pathways for cross border health care.

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> An experience-based evaluation of rare rheumatic patients is more subject to inter-rater as well as to intra-rater variability

- In the absence of agreed guidelines about what information to collect, critical information might be overlooked.

Unexplained Variability:

- may affect health care and lead to poor outcomes,

ORIGINAL ARTICLE

- Lower risk of avoidable hospitalizations at centers that admit large numbers of patients with SLE

- Correlation between in-hospital mortality, physician volume and hospital experience

In-Hospital Mortality in Patients With
Systemic Lupus Erythematosus

Michael M. Ward

Unexplained Variability:

- may affect health care and lead to poor outcomes,

- has an economic impact on the use of resources;

- complicates comparisons among practices.

Recommendations

- Systematically developed statements to help practitioners and patients to make decisions in specific clinical circumstances.

- Guidelines on the items of information to be taken into account when assessing a patient should reduce variability.

- Major difficulties arise in the application of recommendations into clinical practice

ORIGINAL ARTICLE

Rheumatologists' Awareness of and Screening Practices for Hepatitis B Virus Infection Prior to Initiating Immunomodulatory Therapy

JONATHAN G. STINE, OMAR S. KHOKHAR, JOHN CHARALAMBOPOULOS, VICTORIA K. SHANMUGAM, AND JAMES H. LEWIS

Table 1. Questionnaire survey responses*			
Questions and response categories	Yes	No	Unsure
1) Are you aware of any published literature or American College of Rheumatology guidelines recommending screening for hepatitis B infection prior to initiating therapy with:			
Biologic DMARDs	97 (70)	25 (18)	16 (12)
Nonbiologic DMARDs	73 (53)	43 (31)	22 (16)
Steroids	16 (12)	92 (67)	30 (22)
2) Are you aware of drug package inserts recommending screening for hepatitis B prior to initiating drug therapy with:			
Etanercept (Enbrel)	63 (46)	36 (26)	37 (27)
Infliximab (Remicade)	64 (47)	38 (28)	34 (25)
Adalimumab (Humira)	62 (46)	38 (28)	36 (26)
Anakinra (Kineret)	26 (19)	53 (39)	57 (42)
Abatacept (Orencia)	47 (35)	43 (32)	46 (34)
Rituximab (Rituxan)	72 (53)	27 (20)	37 (27)
3) If the drug package insert does not specifically recommend to screen for hepatitis B prior to initiating drug therapy, would you still proceed with screening?	98 (72)	15 (11)	23 (17)
4) Do you routinely screen your patients for hepatitis B infection prior to initiating therapy with:			
Biologic DMARDs	95 (69)	8 (6)	34 (25)†
Nonbiologic DMARDs	58 (42)	13 (9)	66 (48)†
Steroids	9 (7)	64 (47)	64 (47)†

Reasons for low adherence to Recommendations

- Physician's preferences

- Subject of Recommendation

- Inner properties of the recommendations, low feasibility

- The environment and available resources

- **Definition** of existing **guidelines** and **recommendations**; **assess adherence** and **implementation** of their use in clinical practice.

- **Development** of re**commendations/guidelines**.

The case study of Behçet's Disease (BD)

(*Taylor J et al, Interventions for the management of oral ulcers in Behçet's disease. Cochrane Database of Systematic Reviews 2014*)

Research question: to assess the efficacy and safety of 13 drugs/biologicals on pain, episode frequency of oral ulcers and on quality of life in patients with BD.

- **15** randomised trials were conducted, **888** patients involved

- <u>no clear and conclusive evidence</u>

- research gaps identified in these trials due to the content and the methodology (validity of findings), i.e.: heterogeneity of the outcome measurements (nine different oral outcome measurements used)

RARE-Bestpractices platform
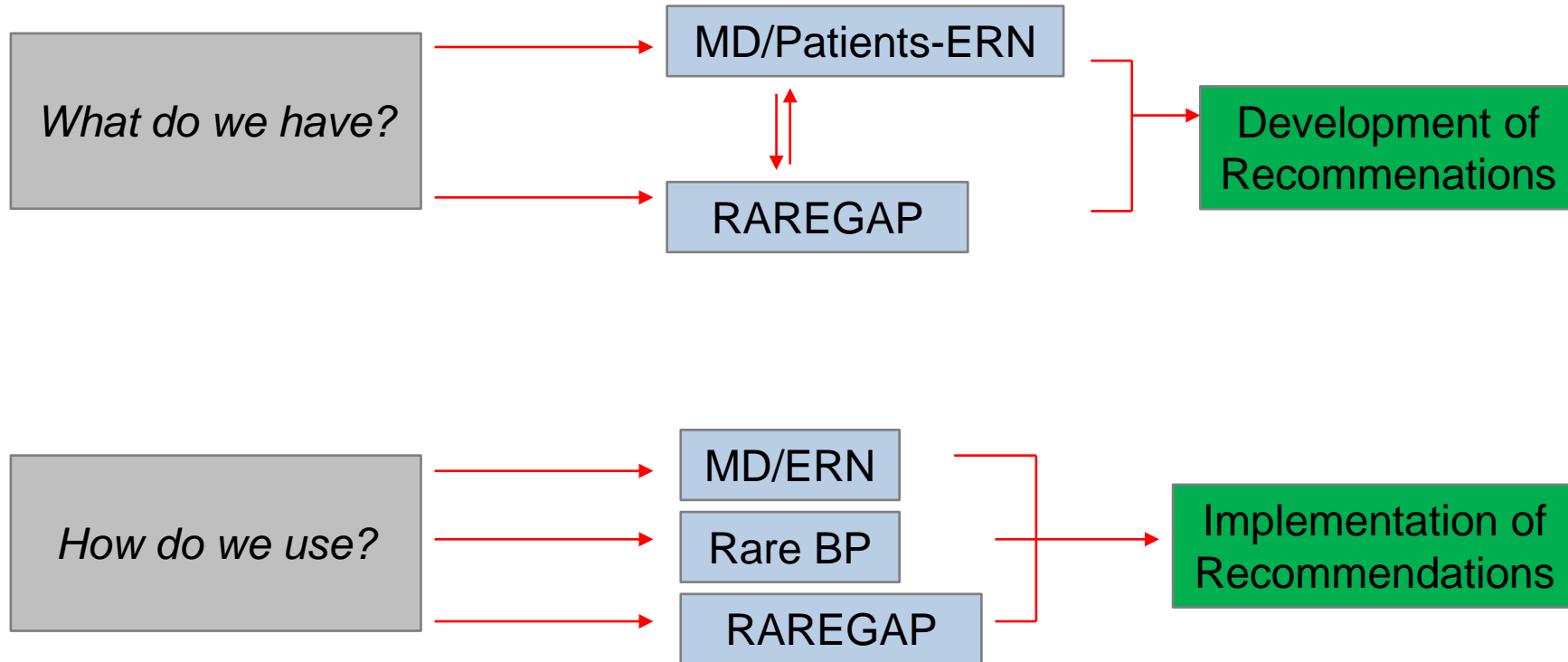the databases, the content they provide and
the sources for content

CLINICAL IMPACT

Definition of existing **guidelines** and **recommendations**

Assess adherence and **implementation** of their use in clinical practice

Development of **recommendations/guidelines**

Clinical impact of
ERN/Rare Best Practices collaboration

What do we have? → MD/Patients-ERN, RAREGAP → **Development of Recommenations**

How do we use? → MD/ERN, Rare BP, RAREGAP → **Implementation of Recommendations**

CLINICAL IMPACT

Definition of existing **guidelines** and **recommendations**

Assess **adherence** and **implementation** of their use in clinical practice

Development of **recommendations/guidelines**

RESEARCH IMPACT

Definition of existing **gap in methodology** (i.e.: creating more homogeneous outcomes)

Planning the **future of reasearch,** aimed at answering to unmet need and pragmatic clinical questions