

Clinical practice guidelines on rare diseases

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Conference on Rare diseases and European
reference networks*

Clinical practice guidelines

- Clinical practice guidelines are systematically developed statements to assist **practitioner** and **patients** decisions about appropriate health care.
- Recommendations are based on **evidence** from rigorous systematic literature review.
- Assessment and opinions of the **experts** of the committees are taken into consideration when writing the guidelines.

Clinical practice guidelines:

- Reduce inappropriate care
- Minimize geographic variations in practice patterns
- Enable effective use of health care resources

Clinical practice guidelines in rare diseases

- The development of guidelines for RD presents many challenges compared to more common diseases.
- This is related to *limited data availability* as well as *health professional knowledge* and *experience* due to low disease prevalence.

State of the art on existing CPGs

Unmet needs on CPGs and on rCTDs

Survey

Integration of ERN in the development of new CPGs

Lay versions of existing CPGs and of State of the art on CPGs

Dissemination of existing guidelines

Educational activities for patients and healthcare professionals

ADAPTE methodology

ERN ReCONNET state of the art on Clinical Practice Guidelines for rare and complex connective tissue and musculoskeletal diseases

State of the art on existing CPGs

Update

Survey

Integration of ERN

Lay versions of existing

Dissemination

Educational activities for

ADAPTE methodology



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Unmet needs on CPGs and on rCTDs

- Clinicians unmet needs: develop guidelines for the treatment for rare disease related manifestations, improvement of adherence to treatment, role of patients, impact of ethnicity
- Patients' unmet needs
 - delay and uncertainty in diagnosis until confirmation by a specialist
 - need for new treatment options, less reliant on steroids and associated with fewer side effects
 - more holistic disease management
 - scientifically validated patient focused guidelines on lifestyle issues

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Unmet needs

Clinicians'
perspectives

Patients'
perspectives

Holistic approach
Lack of guidelines
Need to have defined pathways
Heterogeneity of access care

*Patients as authors of the publication
Best practice in Eurordis ePAGs*

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**RMD
Open**

Rheumatic &
Musculoskeletal
Diseases

Original research

Clinical practice guidelines adherence, knowledge and awareness in rare and complex connective tissue diseases across Europe: results from the first ERN ReCONNET survey

Rosaria Talarico,¹ Diana Marinello,¹ Stefano Bombardieri,² Gerd Burmester,³ Joao Fonseca,^{4,5} Charissa Frank,⁶ Ilaria Galetti,⁷ Eric Hachulla,⁸ Frederic Houssiau,⁹ Ulf Mueller-Ladner,^{10,11} Matthias Schneider,¹² Vanessa Smith,^{13,14} Giuseppe Turchetti,¹⁵ Jacob M van Laar,¹⁶ Ana Vieira,¹⁷ Maurizio Cutolo,^{18,19} Marta Mosca^{1,20}

To cite: Talarico R, Marinello D, Bombardieri S, *et al.* Clinical practice guidelines adherence, knowledge and awareness in rare and complex connective tissue diseases across Europe: results from the first ERN ReCONNET survey. *RMD Open*.

ABSTRACT

Introduction The European Reference Network (ERN) ReCONNET is the ERN aimed at improving the management of rare and complex connective tissue and musculoskeletal diseases (rCTDs) across the European Union (EU). In the mission of ERN ReCONNET, clinical practice guidelines (CPGs) play a crucial role, representing a valid tool towards the

Key messages

What is already known about this subject?

► Clinical practice guidelines (CPGs) have an important role in guiding and supporting the decision-making processes. CPGs cover at present only some rare and complex connective tissue diseases and more efforts

Feedback from HCPs

- *Difficulties and barriers still exist for the use of CPGs in clinical practice*
- Mainly represented by *local legislative restrictions* and *time limitation* during the assessment of patients.
- Proposals: development of *easier and more practical version of CPGs*.

Feedback from patients

- CPGs should be more widely *disseminated* and *adapted* to the different health systems in Europe in order to improve the level of care provided to patients.
- *Patient-friendly versions of CPGs* and *patients' lifestyle guidelines* could play a great role in the process of empowering patients in the management of their disease and to support shared decision with healthcare professionals.

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Recommendation

EULAR recommendations for the management of Sjögren's syndrome with topical and systemic therapies

Manuel Ramos-Casals ,^{1,2} Pilar Brito-Zerón,^{2,3} Stefano Bombardieri,⁴
Hendrika Bootsma,⁵ Salvatore De Vita,⁶ Thomas Dörner ,⁷ Benjamin A Fisher ,^{8,9}
Jacques-Eric Gottenberg,¹⁰ Gabriela Hernandez-Molina ,¹¹ Agnes Kocher ,^{12,13}
Belchin Kostov,^{14,15} Aike A. Kruize,¹⁶ Thomas Mandl,¹⁷ Wan-Fai Ng,^{18,19}
Soledad Retamozo,^{20,21} Raphaële Seror,^{22,23} Yehuda Shoenfeld,^{24,25}
Antoni Sisó-Almirall ,^{14,26} Athanasios G. Tzioufas,²⁷ Claudio Vitali,²⁸
Simon Bowman,²⁹ Xavier Mariette,^{22,23} On behalf of the EULAR-Sjögren Syndrome
Task Force Group

Recommendations

ERN ReCONNET points to consider for treating patients living with autoimmune rheumatic diseases with antiviral therapies and anti-SARS-CoV-2 antibody products

R. Talarico¹, G.A. Ramirez², S.C. Barreira³, C. Cardamone⁴, P. Triggianese⁵, S. Aguilera⁶, J. Andersen⁷, T. Avcin⁸, K. Benistan⁹, G. Bertsias¹⁰, A. Bortoluzzi¹¹, C. Bouillot¹², I. Bulina¹³, G.R. Burmester¹⁴, S. Callens¹⁵, P.E. Carreira¹⁶, R. Cervera¹⁷, M. Cutolo¹⁸, L. Damian¹⁹, E. Della Torre², R. Faria²⁰, J.E. Fonseca³, I. Galetti²¹, E. Hachulla²², L. Iaccarino²³, S. Jacobsen²⁴, N. Khmelinskii³, M. Limper²⁵, D. Marinello¹, A. Meyer²⁶, G. Moroncini²⁷, G. Nagy²⁸, M. Olesinska²⁹, C. Pamfil¹⁹, M. Pileckyte³⁰, M. Pistello³¹, S. Rednic¹⁹, C. Richez³², V.C. Romão³, M. Schneider³³, S. Sciascia³⁴, C.A. Scirè³⁵, G. Simonini³⁶, V. Smith³⁷, A. Sulli¹⁸, C. Tani¹, S.W. Tas³⁸, A. Tincani³⁹, M.C. Vonk⁴⁰, M. Tektonidou⁴¹, M. Mosca^{1,42}

Development of red flags for the diagnosis of
IgG4 related disease

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ERN ReCONNET

LAY VERSIONS

Disease: Systemic Sclerosis

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ANTIPHOSPHOLIPID SYNDROME

1. EULAR recommendations for women's health and the management of family planning, assisted reproduction, pregnancy and menopause in patients with systemic lupus erythematosus and/or antiphospholipid syndrome

Andreoli L, Bertsias GK, Agmon-Levin N, Brown S, Cervera R, Costedoat-Chalumeau N, et al.
Ann Rheum Dis. 2017 Mar;76(3):476-85.

2. [Screening and management of cardiovascular risk factors in systemic lupus erythematosus: Recommendations for clinical practice based on the literature and expert opinion]

Arnaud L, Mathian A, Adoue D, Bader-Meunier B, Baudouin V, Bellizna C, et al.
Rev Med Interne. 2015 Jun;36(6):372-80.

3. Guidelines for immunologic laboratory testing in the rheumatic diseases: anti-Sm and anti-RNP antibody tests

Benito-Garcia E, Schur PH, Lahita R, American College of Rheumatology Ad Hoc Committee on Immunologic Testing G.
Arthritis Rheum. 2004 Dec 15;51(6):1030-44.

4. EULAR recommendations for the management of systemic lupus erythematosus. Report of a Task Force of the EULAR Standing Committee for International Clinical Studies Including Therapeutics

Bertsias G, Ioannidis JP, Boletis J, Bombardieri S, Cervera R, Dostal C, et al.
Ann Rheum Dis. 2008 Feb;67(2):195-205.

5. EULAR recommendations for the management of systemic lupus erythematosus with neuropsychiatric manifestations: report of a task force of the EULAR standing committee for clinical affairs

Bertsias GK, Ioannidis JP, Aringer M, Bollen E, Bombardieri S, Bruce IN, et al.
Ann Rheum Dis. 2010 Dec;69(12):2074-82.

6. Joint European League Against Rheumatism and European Renal Association-European Dialysis and Transplant Association (EULAR/ERA-EDTA) recommendations for the management of adult and paediatric lupus nephritis

Bertsias GK, Tektonidou M, Amoura Z, Aringer M, Bajema I, Berden JH, et al.
Ann Rheum Dis. 2012 Nov;71(11):1771-82.

7. [Recommendations from the Brazilian Society of Rheumatology on the diagnosis and treatment of intestinal parasitic infections in patients with autoimmune rheumatic disorders]

Braz AS, de Andrade CA, da Mota LM, Lima CM.
Rev Bras Reumatol. 2015 Jul-Aug;55(4):368-80.

ERN ReCONNET
WELCO

Welcome to the European
Diseases Website.
An information hub v

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CLINICAL PRACTICE GUIDELINES: WHAT DO YOU NEED TO KNOW



Ilaria Galetti

20th October 2020
15:00 CET



Rosaria Talarico



European
Reference
Network

Connective Tissue and Musculoskeletal
Diseases (ERN hCONNET)

Ilaria Galetti and Rosaria Talarico, Clinical
practice guidelines: what do you need to know

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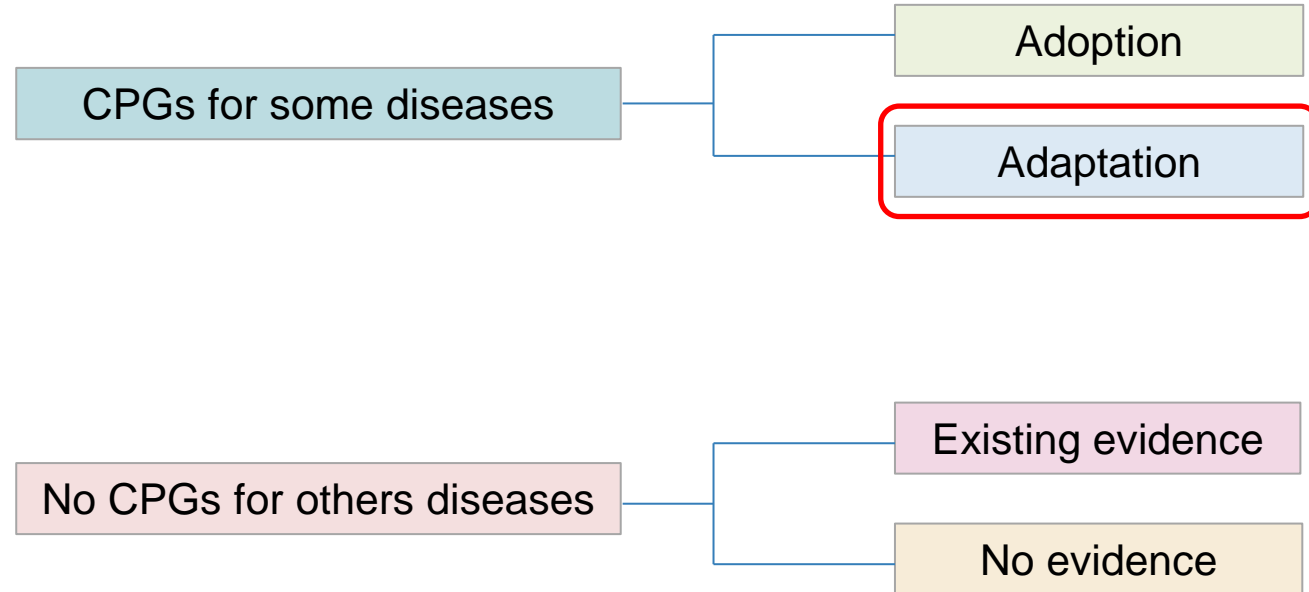
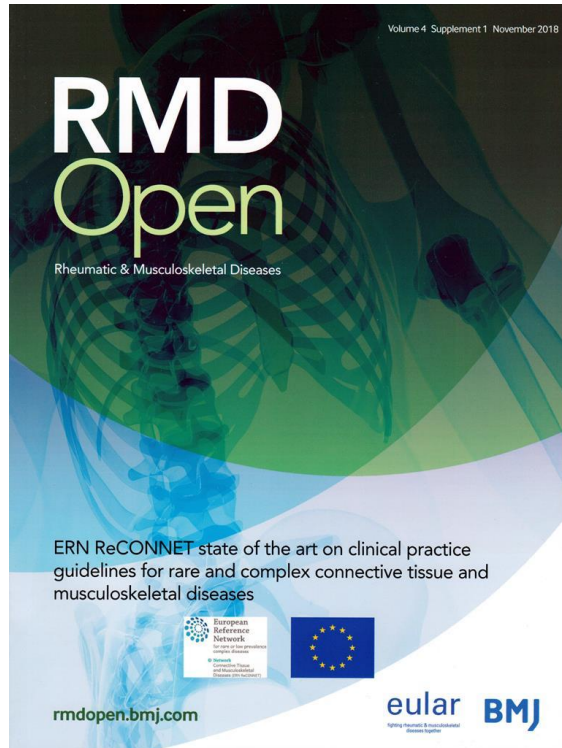
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ERNs and Clinical practice guidelines

- Development of CPGs: evidence AND experts AND patients
- Points to consider
- Implementation of existing Recommendations
- Lay versions: patients
- Adaptation: integration with existing health care systems
- Development of evidence: observational studies, clinical trials, real world evidence, knowledge generation, registries...

Thank you

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