

# PATIENT INVOLVEMENT IN IDENTIFYING UNMET NEEDS ON CLINICAL PRACTICE GUIDELINES



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ERN ReCONNET



European  
Patient  
Advocacy  
Group

# SUMMARY

- ReCONNET Steering Committee (SC) decided to publish a narrative review of existing Clinical Practice Guidelines (CPGs) and/or perform a state of the art (of the existing) CPGs per disease group.
- Proposal made by the ePAGs in the SC to identify the unmet patients' needs in each disease.
- Decided to have clinicians and patients drafting the papers per disease where ePAG would focus on the patients' unmet needs of each disease.
- The papers are published in BMJ – RMD open [https://rmdopen.bmj.com/content/4/Suppl\\_1](https://rmdopen.bmj.com/content/4/Suppl_1)

# SUMMARY

## ERN ReCONNET

### Rare Connective Tissue Diseases

Systemic sclerosis

MCTD

Idiopathic inflammatory  
Myopathies

APL

UCTD

IgG4 Related diseases

Relapsing Polychondritis

### Complex Connective Tissue Diseases

Systemic Lupus Erythematosus

Sjogren Syndrome

### Hereditary Connective Tissue Diseases

Ehlers Danlos Disease

Disease subgroup shall consist of a representative of each Healthcare Provider and a representative of the patient associations

# ReCONNET ePAGs structure

- 1 representative/disease
- 1 representative/pillar (rare, complex and hereditary) and the 3 are SC members
- 1 Senior and 1 Junior Coordinator
- 1 member and 1 alternate in all ERNs ePAGs SC

# ERN ReCONNET ePAG

## Rare CTDs



Ilaria Galetti  
Systemic Sclerosis



Vera Guimarães  
Mixed Connective Tissue  
Diseases

Yves Brun  
Idiopathic inflammatory  
Myopathies

Sander Otter  
Antiphospholipid  
syndrome



Lisa Matthews  
Relapsing Polychondritis

## Complex CTDs



Alain Cornet  
Systemic Lupus  
Erythematosus



Ana Vieira  
Sjögren Syndrome

## Hereditary CTDs



Charissa Frank  
Juergen Grunert  
Ehlers-Danlos Syndromes



## ReCONNET SC Members:



## ReCONNET Sr and Jr coordinators:



Missing ePAGs for Undifferentiated Connective Tissue Diseases,  
IgG4 Related Diseases

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# SITUATION

- Only 4 out of the 10 diseases had previous CPGs (Systemic Lupus Erythematosus, Idiopathic Inflammatory Myopathies, Systemic Sclerosis, Sjögren Syndrome)
- Remaining 6 conditions were lacking CPGs and/or recommendations and were to be reviewed as well.
- A first - In rare Connective Tissue Disorders (rCTDs) papers have not included the unmet needs and taking patient perspectives into account.
- Originally clinicians were the ones in charge of performing the literature review and drafting the papers.
- The ePAGs in SC asked to be involved in the review process to represent the patients' perspective as it may be beneficial to include patients' unmet needs in the papers.

# SITUATION

- Coordinator and SC agreed; ePAGs would identify and draft the patients' unmet needs on CPGs and co-authors the papers.
- The 3 ePAGs in SC were tasked with reviewing the patients' contributions and were also recognised as co-authors in all papers
- There was not an ePAG patient advocate for all the diseases and the ePAG group had to find one.
- For 4 diseases none found on time and the unmet needs section for these diseases was developed by the ePAG patient advocates who are members of ReCONNET SC.
- Most clinicians had never worked with patients on this level.
- 6 diseases had no Clinical Practice Guidelines or pre-existing documentation **THIS WAS ALREADY MENTIONED**

# CONTRIBUTION TO PATIENT ENGAGEMENT AND/OR IMPROVEMENT OF CARE

- ePAG patient advocates **engaged with their wider European patient community** to identify unmet needs. They had to work also for those conditions not represented by a patient advocate.
- All unmet needs identified and written by the ePAG patient advocates were **discussed with the senior and junior clinical coordinators** per disease and the patients
- **Transversal unmet needs** were identified as a result of this exercise.
- The level of patient involvement provided the opportunity to have the patients' voice conveyed at high-level and in a meaningful way help to push for the **development of better standards of care** taking patients unmet needs into account.
- The patients' unmet needs are **acknowledged by the scientific community**, some projects have already started to address some of them, such as certification of the website and therapeutic education.





## REVIEW

Systemic sclerosis: state of the art on  
clinical practice guidelines

Vanessa Smith,<sup>1,2</sup> Carlo Alberto Scirè,<sup>3,4</sup> Rosaria Talarico,<sup>5</sup> Paolo Airo,<sup>6</sup>  
Tobias Alexander,<sup>7</sup> Yannick Allanore,<sup>8,9</sup> Cosimo Bruni,<sup>10,11</sup> Veronica Codullo,<sup>12,13</sup>  
Virgil Dalm,<sup>14</sup> Jeska De Vries-Bouwstra,<sup>15</sup> Alessandra Della Rossa,<sup>5</sup> Oliver Distler,<sup>16</sup>  
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Frederic Houssiau,<sup>23,24</sup> Ulf Mueller-Ladner,<sup>36</sup> Matthias Schneider,<sup>37</sup>  
Jacob M van Laar,<sup>38</sup> Ana Vieira,<sup>39</sup> Maurizio Cutolo,<sup>21</sup> Marta Mosca,<sup>40</sup>  
Marco Matucci-Cerinic<sup>10,11</sup>

## 6

## Systemic sclerosis

## Patients' unmet needs

Patients with SSc experience significant uncertainty concerning SSc-related taxonomy, management (both pharmacological and non-pharmacological) due to lack of (inter-)national harmonisation and standardisation and due to non-existence of overarching evidence-based and consensus-based guidelines for holistic SSc management. Access to uniform information, including knowledgeable HCPs, and management of difficult social interactions and negative emotions are key challenges.<sup>11</sup> Patient education programmes should be promoted.

Besides these, patients with SSc incur considerable costs (eg, non-reimbursement of certain therapies) and experience substantial deterioration in health-related quality of life (HRQoL).<sup>12,13</sup> Additionally, no specific recommendations are at hand regarding non-pharmacological interventions (eg, behavioural/psychological, educational, physical/occupational therapy) to improve HRQoL. However, incentives like the 'Scleroderma Patient centered Intervention Network', which aims to develop, test and disseminate a set of accessible interventions designed to complement standard care to improve HRQoL, are encouraging.<sup>14</sup>

Importantly, patient participation in patient-reported outcome measures, meant to provide insight into the patient condition which is not fully captured by physician-derived assessment tools, has been non-prevalent even though this is paramount to ensure adequate capturing of those experiences most important to our patients.<sup>15</sup>

Last but not least, day-to-day impact of the disease (loss of self-esteem, fatigue, sexual dysfunction, and occupational, nutritional and relational problems) is underestimated.<sup>16,17</sup>

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RMD  
OpenRheumatic &  
Musculoskeletal  
DiseasesEuropean  
Reference  
NetworkConnective Tissue and Musculoskeletal  
Diseases (ERN ReCONNET)

## REVIEW

Sjögren's syndrome: state of the art on  
clinical practice guidelines

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## Patients' unmet needs

This paragraph intends to highlight the unmet needs of the Sjögren's Syndrome European community. The content of this paragraph has been realised by the ERN ReCONNET European Patient Advocacy Group that carefully collected the voices and the points of view of the whole European community of the disease they represent.

Finding a physician that understands Sjögren is still a big challenge nowadays, and this is probably the underlying cause of the most prevalent reported unmet need: delay to diagnosis. Patients are often dismissed because doctors can't relate symptoms, or they are not taken seriously. Consequently, lack of understanding from family, friends and employers paves the way to a heavy emotional burden, not to mention disease progression-related issues.

Besides early diagnosis, delay to treatment is the second most widespread unmet need. It's not clear if doctors don't know how to treat Sjögren's or if it's seen exclusively as a dry eyed and dry mouth disease. Whether we need combination therapies or new therapeutic targets, the fact is effective and specific treatment lacks, and treatments for Sjögren are largely symptomatic. Sjögren's has been an orphan disease in what concerns to specific drugs approval, so establishing rational targets for drug development is a must. Being a highly heterogeneous disease, even at molecular level, shows the need for stratification and tailored treatment strategy. In this concern, genetics must be understood as it seems to play an important role.

Fatigue, pain and cognitive dysfunction, often seen as "benign features", are the cause of greatest patient-reported disability. Understanding how these symptoms truly impact patients' lives and clarifying if depression results from disease activity, or is fatigue related, is a big challenge that urgently needs to be addressed. Disease self-management programme—patient education; lifestyle guidelines—along with non-pharmacological approaches—exercise; meditation; occupational therapies—are interesting resources that lack to be explored and can be key to restore quality of life. There's life beyond the disease and patients deserve to live it at its fullest, but they need to be empowered to know how to manage it in all circumstances.

Finally, more reliable information is needed to address the lack of knowledge from HCPs, public, family, friends and employers.



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Diseases (ERN ReCONNET)

## REVIEW

Ehlers-Danlos syndromes: state of the  
art on clinical practice guidelinesAlberto Sulli,<sup>1</sup> Rosaria Talarico,<sup>2</sup> Carlo Alberto Scirè,<sup>3</sup> Tadej Avcin,<sup>4</sup> Marco Castori,<sup>5</sup>  
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## Patients' unmet needs

The EDS nosology was redefined in 2017 into 13 rare and complex hereditary connective tissue disorders with a prevalence ranging from about 1:5000 to ultra-rare where only few patients or families in the world have been identified. A 14 type was added in 2018.

Although the new nosology has brought attention to EDS, patients still have trouble to find fast access to correct diagnosis and treatment. Not many physicians have been trained to recognise EDS or do not know how to treat it. In many European countries and beyond, there are no diagnostic centres or experts available to patients.

Some of the rarer types can have life-threatening complications and are often only recognised when a (near) deadly event has occurred (eg, in the vascular type of EDS). As there is little or almost no educational information available for healthcare professional and patients, there are many unmet needs in this matter.

Most patients with EDS suffer from generalised joint hypermobility, chronic widespread pain and fatigue. Pain treatment is complex and usually requires guidance of a specialised pain clinic and the support of an integrate rehabilitation programme. Clinical experience suggests that medical marijuana may be a successful alternative to opioids. However, in many countries in the EU, this treatment is not available.

Because of the tissue fragility, conservative treatment is preferred over surgery. To improve daily life functioning, many patients need orthotics to stabilise hypermobile joints, mobility aids, aids for self care and household, etc. Unfortunately, the needs of patients are often misunderstood, because their main problems are 'invisible'. For instance, joint hypermobility is difficult to observe, unless evaluated with specific clinical tests.

At present, EDS is not curable, but only 'treatable'. Patients presenting pain require multidisciplinary care, including pain medication, intensive physiotherapy, podiatry, psychology, occupational therapy and adequate bracing. Often a holistic or alternative approach (eg, osteopathy) is complimentary to normal treatment. Unfortunately, many treatment options are not reimbursed, even when they improve the quality of life of patients with EDS significantly.

At present, a good number of patients are not taken seriously or even accused of hypochondria, Munchausen or Munchausen-by-proxy. As such, psychiatric diagnoses sometimes precede the actual diagnosis. Furthermore, psychological follow-up is sometimes needed, considering the fact that the long road to the correct diagnosis, and correct treatment of the symptoms often contributes to anxiety and depression.

In conclusion, there is a long road ahead for the EDS Community. Many needs are unfulfilled, including access

# SUCCESS FACTORS

1. **STRATEGIC VISION** - The ePAG in SC saw the opportunity to get involved in a new activity that would have a long-term strategic impact in the delivery of care and actively sought to find a way to get patient advocates involved.
2. **Explaining to clinicians involved how they would benefit from networking with patients.** It was the first time that such a gap analysis on CPGs included patients' views on unmet needs.
3. ReCONNET SC ensured with the support and input of the ePAGs involved in SC, that a **maximum patients' involvement** was secured for each paper so that the patients voice was well represented and heard.
4. The ePAG patient advocates did a significant effort to reach out and consult their own patient community for input and feedback contributing to build and **improve the relationships** between the patient advocates and clinicians → mutual respect and appreciation.
5. It has become evident that more people living with rare conditions need to be trained in **advocacy program** and receive support from EURORDIS and other parties such as EULAR to raise the voice of their specific community.

# LESSONS LEARNED

- Through the active participation in ReCONNET SC and disease working groups, the ePAGs were able to fully represent their RD patient community and bring in the patients' perspective. **Action and pro-action are the roots for change**
- **Explaining your ideas** in a respectful and **diplomatic manner** may ensure that you'll be heard and strengthen your influence within the ERN.
- The **ePAGs input** helped build/**improve the relationships with clinicians** bringing mutual respect and appreciation.
- Writing narrative reviews and reviewing CPGs was a **learning process**
- Putting the **spotlight in the patients unmet needs brought acknowledgment** by the scientific community with new projects based on the identified unmet needs published on papers already starting
- **Expanding the number of patient advocates** involved to fully represent all diseases was a challenge; some diseases were not represented and we had to identify new ePAG.



# LESSONS LEARNED

- It was very **difficult to research *new diseases*** and fully represent these communities.
- More people living with rare conditions should also be **included in training and advocacy program.**
- Activities as these, with a high potential impact on improving standards of care, will **bring your team closer together.** However, someone or some people will need to coordinate the group and work to ensure optimal teamwork
- As a result of the ePAG work and input of these papers, everyone in ReCONNET agreed that **clinicians will wait** with developing patient pathways until the ePAG have developed their own to be used as basis.

# THANK YOU

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