European Reference Network | Connective Tissue and Musculoskeletal Diseases (ERN ReCONNET)

3rd ERN ReCONNET

Plenary Meeting Report

European
Reference
Network
for rare or low prevalence
complex diseases

Network
Connective Tissue
and Musculoskeletal
Diseases (ERN ReCONNET)

13th – 14th February 2019

Scuola Superiore Sant'Anna, Pisa, Italy

Share. *Care*. Cure.

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Introduction

This document contains the report on the third ERN ReCONNET plenary meeting that took place on 13th and 14th January 2019 in Pisa (Italy), at the Scuola Superiore Sant'Anna.

The meeting focused on the following activities:

- ✓ Report and overview of the work done in the 2 years;
- ✓ Identification of Tasks Leaders;
- ✓ Steering Committee meeting;
- ✓ Planning of the next steps.

Participants: Tobias Alexander, Laurent Arnaud, Tadej Avcin, Chiara Baldini, Simone Barsotti, Lorenzo Beretta, Lara Bloom, Stefano Bombardieri, Alessandra Bortoluzzi, Sara Cannizzo, Marco Castori, Lorenzo Cavagna, Ricard Cervera, Alain Cornet, Maurizio Cutolo, Virgil Dalm, Alessandra Della Rossa, Hervé Devilliers, Meryem-Maud Farhat, João Eurico Fonseca, Francisca Fontes, Charissa Frank, Andrea Gaglioti, Ilaria Galetti, Juergen Grunert, Serena Guiducci, Vera Guimarães, Eric Hachulla, Luca Iaccarino, Ramona König, Thomas Krieg, Maarten Limper, Valentina Lorenzoni, Diana Marinello, Marco Matucci Cerinic, Alain Meyer, Pia Moinzadeh, Silvia Morlino, Marta Mosca, Cecilia Nalli, Ilaria Palla, Micheline Pha, Salvatore Pirri, Simona Rednic, Vasco Romão, Maria José Santos, Matthias Schneider, Carlo Alberto Scirè, Vanessa Smith, Alberto Sulli, Delfien Syx, Rosaria Talarico, Farah Tamirou, Chiara Tani, Domenica Taruscio, Anna Viola Taulaigo, Simone Ticciati, Angela Tincani, Leopoldo Trieste, Isotta Triulzi, Giuseppe Turchetti, P. M. Van Hagen, Ana Vieira, Jeska K. De Vries-Bouwstra, Margherita Zen.

Day 1, 13th January 2019

Welcome speeches

Marta Mosca, coordinator of the ERN ReCONNET, has briefly welcomed the participants, providing an overview the main achievements of the second year and introducing the main objectives and the agenda of the 2 days meeting.

Update on patients' point of view from ePAGs

Ilaria Galetti, patient representative in the ERN ReCONNET European Patients Advocacy Group, highlighted the importance and the crucial added value of the strong collaboration already established with the Network: creating one strong voice to provide rCTDs patients with a better healthcare within Europe. The ERN ReCONNET ePAG includes at present 7 advocates that represent the majority of the diseases of the Network:

- Alain Cornet (Systemic lupus erythematosus- SLE)
- Charissa Frank (Ehlers-Danlos Syndromes - EDS)
- Ilaria Galetti (Systemic Sclerosis - SSc)
- Juergen Grunert (Ehlers-Danlos Syndromes -EDS)
- Vera Guimaraes (Mixed Connective Tissue Disease - MCTD)
- Lisa Matthews (Relapsing Polychondritis -RP)
- Ana Vieira (Sjogren's Syndrome - SS).

In order to ensure that the voice and needs of rCTDs patients are taken into consideration in the activities of the Network, the ePAG have established a specific structure within the group, identifying a Senior and a Junior Coordinator (Juergen Grunert and Ilaria Galetti) and appointing one ePAG for each Work Package.

Ilaria Galetti has also outlined a document recently developed by EURORDIS, the "Recommendations on Integration of ERNs into National Health Systems" and summarised the main results obtained thanks to the contribution of the ePAG to the ERN ReCONNET:

- Identification, collection and integration of patients' unmet needs in the State of the Art on Clinical Practice Guidelines (CPGs) supplement;
- Revision and dissemination of surveys;
- Participation to the EURORDIS activities developed for ePAG;
- Organisation of regular webconferences and participation to meetings;
- Dissemination of ERN ReCONNET activities in national and international conferences;
- Identification of new ePAGs to represent all the diseases of the ERN ReCONNET.

New activities are also planned, such as the translation into lay language of the State of the Art on CPGs for patients, the creation of a patients journey for rCTDs and the development of an "ERN ReCONNET endorsement" for scientifically relevant websites.

The last activity was better explained by *Alain Cornet*, ePAG for SLE, who conceived the proposal of designing a "quality information stamp" of the ERN ReCONNET for websites that concern rCTDs. It is well known that the majority of patients search for information on their disease(s) on the web. Unfortunately, it is also well known, that information found on the internet are often low quality, counterproductive and anxiety-generating. The proposal aims to address the need of patients of having high quality information on their disease(s) also on the internet and avoid the generation of fake news. To do that, Alain proposes the creation of a Task Force that will develop guidelines and checklists to review and endorse websites, that will initially focus on a pilot endorsement for SLE website. The proposal was very well received by the participants and has been further discussed during the second day.

Next call for new HCPs and Affiliated Partners

The first roll-out of the next call for the inclusion of new Healthcare Providers (HCPs) into existing ERNs will be launched within the end of the first semester of 2019.

As far as the identification and selection of Affiliated Partners, Member States not represented in a specific ERN will designate Affiliated Partners taking into account their individual situation and planning. The minimum recommended criteria for the designation of Affiliates Partners have been defined by ERN Board of Member States (BoMS), the documentation is available on the ERN website of the European Commission (EC). The ERN ReCONNET National Representative have been identified in the last months and will serve as expert contacts for the national health authorities and eventually involved in the endorsement process. At present, one Affiliated Partner has been identified for Austria.

ERN ReCONNET National Expert Contact for national health authorities

Country	ERN national expert contact for national health authorities
Belgium	Vanessa Smith
France	Eric Hachulla
Germany	Matthias Schneider
Italy	Marta Mosca
Netherlands	Jacob van Laar
Portugal	João Cabral da Fonseca
Romania	Simona Rednic
Slovenia	Tadej Avcin

The criteria for the selection of new HCPs will remain the same established in 2016, as indicated by the European Commission.

ERN Continuous Monitoring

In order to demonstrate the added value of ERNs, the EC has identified a specific process for quality assessment, monitoring and evaluation of ERNs. *Sara Cannizzo* introduced continuous monitoring of ERN that will measure the progress of ERNs using 18 common indicators, that were agreed by the ERNs BoMS and the ERN Coordinators Monitoring Working Group (WG) and DG SANTE.

The 18 common indicators include the monitoring of the level of functional collaboration between HCPs and coverage of involved countries in Europe, the improvement of care and treatment for people with rare diseases or complex conditions, education and training. A dedicated IT System will be launched by the EC and during 2019 each ERN will develop their specific set of indicators (addressing clinical and not clinical outcomes).

In 2018, Hôpital Pitié-Salpêtrière (Paris) has been selected for a technical assessment and the University Medical Center Utrecht (Utrecht) has been identified for both a technical assessment and an on-site audit.

Maarten Limper summarised the successful process for University Medical Center Utrecht, carried out by the Andalusian Agency for Healthcare Quality (ACSA).

Clinical Patients Management System (CPMS): how it works and why it is important

The Clinical Patient Management System (CPMS) is a secure web-based application to support European Reference Networks (ERNs) in the diagnosis and treatment of rare or low prevalence complex diseases or conditions across national borders. The CPMS enables clinicians and researchers to enrol patients to collaborate actively and share patient data within and across ERNs using comprehensive data models.

CPMS is a revolutionising tool to provide capacity for cross-border virtual consultations, and research activities.

Marta Mosca and *Simone Barsotti* highlighted the importance of the CPMS that could become an unprecedented opportunity for physicians and patients, beyond the existing issues.

Within the Network, some cases have already been discussed, and the Coordinators reported on one specific case that was particularly helpful for outlining the main challenges and opportunities of the CPMS:

Opportunities	Challenges
✓ Ensure protection of the patient's personal data (according to EU-GDPR)	✗ The registration is not user friendly
✓ Easy way to share knowledge and expertise across the Europe	✗ Not all HCPs are registered
✓ Possibility to involve experts from other ERNs and guests from non-ERN HCPs	✗ Patients reported that the "informed consent" form is unfriendly and difficult to read
✓ May allow immediate actions to improve the clinical course of the patient	✗ Familiarising with the Platform can be time consuming
✓ Production of an outcome document	
✓ Immediate visualization by all the panel members of the answers	
✓ Possibility to share documents, images, directly from the platform	

In order to address the challenges and implement the use of the CPMS, an ERN ReCONNET Helpdesk has been launched thanks to the support of the EC funding and is now functioning with Simone Barsotti as the main clinician involved and an IT expert is being recruited. Simone Barsotti provided an overview of the main features and tools of the CPMS and the role of the ERN ReCONNET Helpdesk, that are summarised below:

- Preparation of 'virtual' advisory boards of medical specialists across different disciplines to review a patient's diagnosis and treatment.
- Monitoring of the participation of Users, Healthcare providers Organizations, and Centres in the ERN (Reports on Centre activity, number of Cases, HPs and access to the system by period).
- Monitoring of the number of patients in the system.
- Creation of "Cohorts" in the system allowing classification and reporting on patients by disease and treatment groups.
- Key Performance Indicators data and information to manage and oversee the ERN.
- Management of the requests and granting or denying the access.

Simone Barsotti emphasized that the CPMS that the CPMS is useful for discussing difficult cases at referral centres, but mostly should be used by centres outside the ERN, where the expertise is needed. Therefore, it is particularly important that each HCP is registered and participate to the CPMS activities.

Report on EU calls and activities of the ERN Coordinators Group

Sara Cannizzo summarized the main EU calls and related activities for the co-funding the coordination and management of ERNs, the INEA calls for co-funding the implementation of CPMS and ERN Collaborative Platform (ECP), and the EU calls for funding research in RDs (the European Joint Programme for Rare Diseases).

Diana Marinello outlined the role of the ERN BoMS, ERN Coordinators Group and the ERN Team within the EC. In addition, an overview of the activities of the ERN Integrated Working Groups, composed by the ERN Coordinators, the representatives of the ERN BoMS and DG SANTE has been provided. The ERN ReCONNET Coordinator is involved in 2 WGS: the WG on Knowledge generation and Clinical Guidelines and the WG on Research. The Research WG has recently convened in Brussels to discuss the development of the ERN Research agenda and the Knowledge generation and Clinical Guidelines organised a meeting on 18th February.

CPGs (ADAPTE and AGREE II)

Alessandra Bortoluzzi, *Carlo Alberto Scirè* and *Rosaria Talarico* presented the ADAPTE project that will be starting in the next weeks.

The ADAPTE process provides a systematic approach to adapting Clinical Practice Guidelines (CPGs) produced in one setting for use in a different cultural and organizational context. The adaptation process consists of three main phases, each with a set of modules.

1. *Set-up Phase:* Outlines the necessary tasks to be completed prior to beginning the adaptation process (e.g., identifying necessary skills and resources).
2. *Adaptation Phase:* Assists users through the process of selecting a topic to identifying specific health questions; searching for and retrieving guidelines; assessing the consistency of the evidence and the guideline quality, currency, content, and applicability; decision making around adaptation; and preparing the draft adapted guideline.
3. *Final Phase:* Guides the user through the process of obtaining feedback on the document from stakeholders impacted by the guideline, consulting with the developers of source guidelines used in the adaptation process, establishing a process for the review and updating of the adapted guideline, and creating a final document.

Based on the results obtained in the State of Art on GPG, the ADAPTE process will involve IIM, SLE, SS and SSc. For each disease a panel of experts will be created, and will be composed of:

- Members of the Steering Committee
- Fellows
- HCP Representatives (one per each country covered by ERN ReCONNET)
- Patients Representatives
- Methodologists
- Policy/administrative experts
- Health economist
- External reviewers

The composition of the panels will be further discussed during the Steering Committee meeting and during the second day of the meeting.

Education

Meryem-Maud Farhat and *Eric Hachulla* presented the results of the Therapeutic Patient Education (TPE) survey that was developed in two different versions: one for HCPs and one for patients.

HCPs version

The main aim of survey was to evaluate what is required to ensure that all patients have access to TPE and to assess the existing TPE practices and needs within EU. Thirty-three responses were collected and the majority of them reported that no staff in their HCP is trained for TPE and all of them would like to receive training on how to perform TPE. The main message collected is that more than 85,3% of responders would like to see the practice standardized at European level.

Patients version

The patients' version of the survey was translated into 9 different languages (French, English, Dutch, Belgian, Romanian, German, Slovenian, Italian and Portuguese) thanks to the huge contribution of the ePAGs that reviewed all the versions. The total answers received is 1907 and the vast majority of them (72%) have never heard of TPE, but would like to take part to a TPE programme. Among the responders that had already heard of TPE, 67% were never invited to a TPE training programme, while the responders that took part to TPE programme were glad to have participated, stating that they also have improved their knowledge on the disease. A "wish list" on patients' education has been drafted and includes the need to be educated on "daily life with the disease, symptoms and treatments, and on the impact of the disease on family and social life". Those needs could be address in the activities of the ERN ReCONNET in the WP 8 – Education.

Maurizio Cutolo has integrated the discussion on Education, providing an overview of the possible synergies with EULAR, underlining the priority to focus on EU registries on rare CTDs since this represent one of the most important mission of the ERN ReCONNET, and also that is a crucial unmet need.

Steering Committee meeting

At the end of the first day of the ERN ReCONNET meeting the Steering Committee convened to discuss the approval of ADAPTE protocol, the role of the external experts, the eligible dates for the next meeting at EULAR congress.

Day 2, 14th January 2019

Report on Steering Committee decisions, Board of Network discussion and approval of SC proposals

Marta Mosca summarized the decisions of the Steering Committee about the ADAPTE protocol, the activities on diseases not included in the ADAPTE process, the external experts and the meeting at the EULAR congress. In order to improve the activities of the network, Marta Mosca proposed to organize the Work packages into Tasks, identifying a Task leader for each task. Each Task leader will coordinate the activities of the task, will create a proposal on how to develop the related results and deliverables within the deadlines foreseen in the project, and will be responsible for the smooth communication among the members of the group and will report to the Network Coordinator. The different tasks will be managed using the ERN Collaborative Platform – ECP in the related Forums (already available).

Task N.	Task Title
Task 1	Use of CPMS
Task 2	ADAPTE
Task 3	Minimum data sets for the collection of clinical data
Task 4	Patients pathways
Task 5	Recommendations/CPGs development
Task 6	Educational webinars for health care professionals
Task 7	Formal educational activities (CME) for healthcare professionals
Task 8	Educational activities for patients, families, patient representatives and patients’ organisations
Task 9	Fellowships for physicians and nurses in ERN expert centres
Task 10	ERN endorsed/produced disease or treatment specific leaflets
Task 11	Website approval
Task 12	Transitional care
Task 13	Research

Task 1 Use of CPMS

The first Task is focused on the use and implementation of the CPMS. The task leader is AOUP in the person of Simone Barsotti. The objectives of Task 1 are the identification of country specific contact points, that would promote CPMS use in their country and provide local support for HCPs. The activities of the country-specific support team would be (1) involvement of multidisciplinary teams at each HCP, (2) preparation and implementation of a list of experts signed in the CPMS, (3) promotion of the use of the CPMS, (4) scheduling of training webinars for the use of CPMS, (5) working in strict contact with the task leader. The country specific support teams are:

- Belgium: V. Smith
- France: A. Meyer
- Germany: M. Schneider
- Italy: AOUP with the support of L. Cavagna
- Portugal: F. Fontes
- Romania: S. Rednic
- Slovenia: T. Avcin
- The Netherlands: J. van Laar

Task 2 ADAPTE

Task 2 involves all activities related to the ADAPTE project that has been outlined during the first day. The composition of the ADAPTE panels has been outlined for each of the 4 rCTDs that will be involved in ADAPTE. Besides the fixed Steering Committee, that is involved in each panel, the HCPs and patients have identified their contribution to the panels based on their expertise and on their availability. The work on ADAPTE will start at the beginning of February with dedicated webconferences with the Panel Members.
Task Leaders are the methodologists involved in ADAPTE (A. Bortoluzzi, R. Talarico and C. A. Scirè).

Task 3 Minimum data sets for the collection of clinical data

The objectives of the Task 3 are the identification by expert opinion of a minimum data set for the collection of clinical data and the creation of a clinical chart which could be the base for a future registry development. For SLE, C. Tani has volunteered to coordinate the work with the support of Düsseldorf, Padova, Berlin and Brescia. For SS, C. Baldini with coordinate with the support of A. Vieira and Lille. For the other diseases, the Team of Taks 3 will be better outlined.

Task 4 Patients pathways

The objective of the Task 4 is the definition of patients' pathways based on expert opinion and existing literature/recommendations/CPGs. At this stage, we would like to postpone the development of pathways to year 4. The proposal for year 3 would be to identify red flags for suspicion of a disease. The work of this Task will be performed by the team of Task 3.

Task 5 Recommendations/CPGs development

The objectives of the Task 5 are the endorsement of existing recommendations/clinical practice guidelines, the development of new recommendations/clinical practice guidelines, and of new patients' guidelines. Ilaria Galetti and Marta Mosca will be Leader of the activity regarding patients' recommendations while a Team will be established for the endorsement activity.

Task 6 Educational webinars for health care professionals

The objectives of the Task 6 are the development and the realisation of educational webinars specifically designed for health care professionals. The Task leader is Vanessa Smith, with the collaboration of ePAGs. For this Task a list of topics to be discussed and a list of speakers available for the webinars realisation will be drafted. In addition, a calendar dedicate to the webinar schedule will be prepared.

Task 7 Formal educational activities (CME)

The objectives of Task 7 are to establish a procedure to connect ERN ReCONNET with existing (or to organise new) educational activities. The Task leaders are Eric Hachulla and Maurizio Cutolo.

Task 8 Educational activities aimed at patients, families, patient representatives and patients' organisations

The objectives of Task 8 are the development and realisation of educational activities specifically designed for patients, families, patient representatives and patients' organisations. The Task Leaders are Alain Cornet and Ana Vieira, with the support of Eric Hachulla for the definition of the best subjects and modalities to deliver educational activities.

Task 9 Fellowships for healthcare professionals ERN expert centres

The objective of the Task 9 is to establish a procedure in order to organise training and host fellows in the ERN Centres. A list of centres available to host health care professionals from centres inside and outside ERNS will be created, and dedicated procedures at each center to facilitate the process will be established.

Task 10 ERN endorsed/produced leaflets

The objectives of Task 10 are the prioritisation of subjects, the identification of existing leaflets and the definition of a team to merge or develop leaflets. Task leaders are the ePAGs, with the collaboration of Joao Fonseca that has volunteered to support in this Task.

Task 11 Websites approval

The objective of Task 11 is to develop the assessment procedures for websites, prioritising the main topics and drafting a protocol. The Task leader is Alain Cornet, with the collaboration of C. Tani, M. Schneider, M. – M. Farhat and G. Turchetti.

Task 12 Transitional care

The objective of Task 12 is to establish a collaboration with ERN RITA in order to develop transversal actions on transitional care. The Task leaders are Tadej Avcin, Micheline Pha, Joao Fonseca and Virgil Dalm.

Task 13 Research

Each HCP can propose to the Network to participate or create research projects. The ERN ReCONNET can endorse research projects by submitting a request to the Steering Committee that will convene and agree on the endorsement.

Research projects & surveys

Chiara Tani proposed to the Network a research project on vaccinations in SLE and *Salvatore Pirri* presented a research project on medication adherence in patients with rare and complex autoimmune rheumatic diseases. The main objectives of the project are: to perform a literature review on medication adherence interventions and factors that influence long-term drugs and life-style compliance in patients with rare and complex connective tissue and musculoskeletal diseases, and to measure the medication adherence rate of the patients thought the ERN ReCONNET network.

Diana Marinello reported on ERN ReCONNET surveys that were developed in the second year.

The survey on eHealth tools was created to assess the level of awareness on e-Health tools and to identify unmet needs about existing tools in the framework of the ERN ReCONNET. The results of the survey highlighted the following key messages:

- low usage and awareness of e-Health tools developed for rCTDs;
- need to develop tools useful for the management of the disease both for HCP and for patients;
- App/website would be the most preferred tools;
- Main items to be addressed are clinical tools, disease information and treatment management (calendar, reminders etc).

The survey on the identification of ethical, legal and privacy issues related to the management of rCTDs and cross-border healthcare has been developed within the WP9 - Ethical and legal issues, and privacy. It aims at identifying and developing strategies to overtake the barriers related to cross border health data exchange and patients' movement. It has been realised in two main versions, for HCPs and for Patients and the version for patients was translated into 6 (Dutch, French, German, Italian, Spanish and Portuguese).

In addition, a survey dedicated to our ePAG was created to collect their views, feedbacks and suggestions on the consent form that has been developed by the European Commission for the enrolment of patients in the ERN Clinical Patient Management System. The results will be published in the ERN ReCONNET website.

Rosaria Talarico presented the survey on Economic and organizational issues on ERN ReCONNET rare diseases, with the aim of mapping the current pathways that patients with rCTDs follow from the referral to the follow up in each official Healthcare Provider of ERN ReCONNET in each disease covered by the ERN. The survey will be launched in the next days.

Next events

The next ERN ReCONNET meeting is planned during the 2019 EULAR Annual European Congress of Rheumatology that will take place in Madrid from 12th to 15th June 2019.