Continuous Monitoring of ERNs

ERN Continuous Monitoring and Quality Improvement System (ERN CMQS)

Set of ERN core indicators (18)

Version V.7

ERN Continuous Monitoring Working Group of the ERN Coordinators Group & the Board of Member States

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History of changes

Version	Date	Change	Pag
V.1	December 2018	▪ Initial version	
V.2	February 2019	▪ Typos correction	
V.3	July 2019	▪ Update-Clarifications	
V.4	July 2019	▪ Update-Clarifications	
V.5	July 2019	▪ Update-Clarifications	
V.6	July 2019	▪ Update-Clarifications	
V.7	September 2019	▪ Update-Clarifications	

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Introduction

The ERNs' legal framework sets out the objectives, principles and criteria of the ERNs and defines the general implementation process including the assessment, approval and evaluation of the ERNs. Once positively assessed and approved, the ERNs are expected to perform and fulfil their goals and criteria and to be evaluated at least every five years.

However, all actors (Member States, ERNs and European Commission) have identified the need to establish a solid and valid continuous monitoring and assessment system of the ERNs to allow a closer follow up of the activities performed by the networks. This system should help to build a quality improvement system, to define appropriate outcomes of the ERNs, to identify areas of success and potential pitfalls and to demonstrate the value of the ERNs, ultimately learning from the experience.

The process to set up such a monitoring and information system involves a huge challenge both at organisational and technical level.

It is important to define a clear strategy to inform Member States health authorities, health care providers, patients and other stakeholders and the public in general, on how the ERNs' monitoring and assessment system and reporting activities are likely to be developed over the next few years.

Following this initial proposal, a fruitful and extensive discussion was held during one year (from April 2017 to April 2018) that allowed the Working Group to exchange views and agree on a methodological approach for building the performance indicators and endpoints (in the case of the outcomes) and at a later stage, to pilot and validate a functional monitoring system.

It was considered that to develop and implement a robust ERN monitoring and assessment system it was important to look at 4 dimensions:

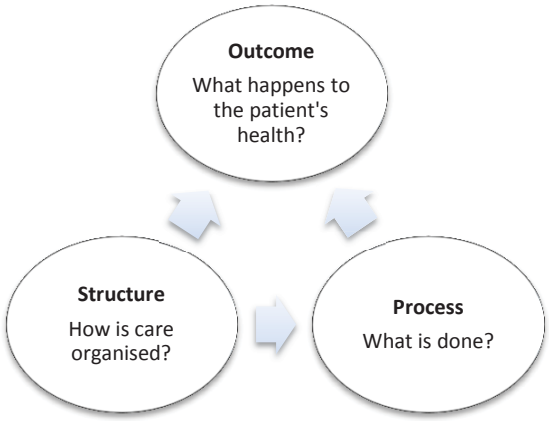
1. Development of a workable continuous monitoring system of the ERN activities which can be utilised across all ERNs.
2. Periodical self-assessment and reporting of the activities of the ERNs and HCPs (similar to the Assessment performed at the initial stage) to the European Commission and the Board of Member States for ERNs;
3. Stronger involvement of Member States in the assessment of their national HCPs wishing to participate in, or participating already in related ERNs to ensure they have or maintain the required levels of expertise
4. Complementary assessments, when considered necessary, by third parties (IABs).

Several actors including patients and other stakeholders were involved in the above dimensions, namely the ERN coordinators, the ERN Board of Member States (BoMs), representatives of EURORDIS and of the Joint Action on Rare Diseases and the European Commission (DG SANTE) providing secretariat for the whole process.

The proposal below supports points 1 and 2 and provides a conceptual framework to carry out continuous monitoring of ERNs by identifying common indicators to all the networks based on the Donabedian model of

structure, process and outcome. Where possible, ERNs should focus on outcome measures which are able to demonstrate that the ERNs have improved the quality of diagnosis, care and treatment. Each ERN will also need to include indicators specific to their ERN and related to the conditions that they each address. Individual indicators will need to be discussed internally within each ERN, with patients and with the ERN coordinators in order to reach agreement on these.

Figure 1: Donabedian model



Why do we need a continuous monitoring system for ERNs

The lifecycle of an ERN follows an annual PDSA model: Plan, Do, Study, Act. Following an initial Plan resulting in the implementation of the ERNs (Do), study, through continuous monitoring is a crucial next step, allowing for timely identification of successes and failures in the system and the opportunity to Act upon the areas requiring improvement before starting a further PDSA cycle.

A monitoring system for ERNs would:

- Provide transparency and reassurance to the rare disease patient community and the public of the expertise within the networks, that care is safely delivered and that there is improved access to quality of diagnosis, care and treatment
- Help ensure consistency across assessments of the Networks and Healthcare providers, support the self-assessment process and promote ongoing quality improvement.
- Show Member States and legislators that the ERNs benefit patients (accountability)
- Allow for timely identification of areas for improvement
- If necessary, foster organisational change or adjustments in strategy
- Promote patient empowerment: when information is released, citizens use it and can make more educated choices
- Request the further support of Member States to the ERNs' system when it is not possible to meet objectives due to lack of resources

Other very important areas that have been proposed, like the holistic care approach to

the patients, although very important, would not be feasible and would challenge the ERNs system and in particular to the measurement capacity of their activities or outcomes due the exclusive national competences in most of the elements related with holistic care.

ERN goals - Identifying common objectives

Legal framework for ERNs

The Directive 2011/24/EU provides a legal framework within the European Union to facilitate cross-border care. Article 12 requires the European Commission to support the Member States in the establishment of the ERNs. As stated in the article, ERNs should have at least 3 of 8 proposed objectives (p.18).

Intervention areas and objectives of ERNs

The overarching objective of European Reference Networks is that patients have an improved access to quality diagnosis, care and treatment.

In order to design a monitoring system that answers this general objective set out in the Directive and consequently to the aims of the ERNs, a review of 10 ERN applications and their respective FPAs was performed. The many activities that need to be managed in order to deliver the ERN objectives were then grouped into 7 ''intervention areas'' and specific objectives, each of which address a part of the general objectives imposed by the Directive.

Table I: ERNs intervention areas' and specific objectives

1. **Intervention area: General organisation and coordination**

   - Objective 1: To ensure that ERNs are operational and successfully carry out their organisational activities

2. **Intervention area: Patient Care**

   - Objective 2: To improve access to clinical advice, diagnosis, treatment and follow-up of patients within the ERNs. Geographical and disease coverage

3. **Intervention area: Multidisciplinary approach and sharing of knowledge within the ERN**

   - Objective 3: To optimise patient outcomes by combining skills of healthcare professionals involved and resources used

4. **Intervention area: Education and Training**

   - Objective 4: To increase capacity of professionals to recognize and manage cases of rare or low prevalence complex diseases and conditions within the scope of the ERN

5. **Intervention area: Contribution to research and innovation**

   - Objective 5: To reinforce clinical research in the field rare diseases and complex conditions by collecting data and carrying out collaborative research activities

6. **Intervention area: Clinical guidelines**

   - Objective 6: To ensure that all patients referred to ERNs have access to high quality healthcare services

7. **Intervention area: Communication and dissemination within the scope of the ERN activities**

   - Objective 7: To guarantee that knowledge and expertise is spread outside the ERN so that more people can benefit from the ERN activities.

The above areas also bear a strong resemblance to the 9 operational criteria of the Assessment Manual of the ERNs.

Proposed indicators for monitoring the ERNs

This set of key performance, structure and outcomes indicators represents one of the four strands of work to be developed to implement the future ERN Continuous Monitoring and Quality Improvement System (ERN CMQS):

> I.- *Continuous monitoring of the Common Core set of ERN 18 Indicators (common to all ERNs)*
>
> *II.- ERN specific set of indicators (by ERN). Including Network specificities and addressing outcomes (clinical and not clinical)*
>
> *III.- ERN extended set of indicators (HCP indicators applications 2016). Periodical self-assessment and reporting of the ERNs and HCPs*
>
> *IV.- External (MS or third parties) validation of HCP fulfilment of the criteria (specific criteria) for healthcare providers defined in the HCP membership applications (2016)*

The framework below presents the ERN objectives and indicators for the first strand: monitoring ERN performance. The definitions of the indicators aim to enable an assessment of any maintenance, improvement or deterioration in relation to the objectives of the ERNs. Furthermore, they aim to facilitate accurate reporting to healthcare authorities, patients, and healthcare providers and clinical and research experts.

A stable set of key performance and outcome indicators can be used to identify opportunities for improvement of the ERNs, and will help ensure cohesion across the EU health care system.

Working procedures and milestones for the definition of the ERN key indicators:

The European Commission presented a paper on indicators at the first meeting of the ERN Coordinators on 26 April 2017, Brussels, Belgium. This paper was compiled using the monitoring information given by the ERNs in the application process. The Commission highlighted the importance of the development of a robust and valid Monitoring and Assessment system of the ERNs, and stressed that the first goal would be to develop a common set of indicators for the whole ERN system.

After an intensive review and discussion process, a total number of 41 indicators were selected initially and agreed by the ERN CG on 5th March 2018. This set of indicators was also presented on the 6th of March to the ERN Board of MS for further agreement.

Both the Board and ERN CG decided to merge both groups, with the Commission acting as Secretariat, in order to reach a final set of core indicators and to define a roadmap for the implementation of the Continues Monitoring System.

In May and June 2018 the secretariat organised virtual meetings of the merged group on ***ERN Continuous Monitoring Working Group of the Member States and the ERN Coordinators.*** Integrated by five Member States (AT –chair- FR, ES, UK, NO) and five ERNs chaired by eUROGEN (CRANIO , ERKnet , eUROGEN , TRANSPLANTCHILD, VASCERN). A representative of the JA on RARE DISEASES and a representative of EURORDIS participated as invited stakeholders with relevant knowledge.

Based on the quality assessment of the initial set of indicators, the WG ended up with a reduced list of 18 Core indicators to be **finally agreed by the ERN Board of MS and the ERN Coordinators Group in September 2018.**

Rationale and methodology for the selection of indicators for the continuous monitoring of the ERNs

The ERNs need to demonstrate that the networks are delivering services and functioning, but simultaneously – and of particular importance, in terms of longevity - are **adding value** compared to what exists. The indicators have therefore been chosen with regard to specifically being able to capture the added value following the establishment of the ERNs. This means that the defined indicators should reflect the level of functional collaboration between European healthcare providers and coverage of involved countries in Europe; level of patient empowerment, contribution and satisfaction; level of knowledge generation through research activities.

Ultimately, this serves to improve care and treatment for people living with rare diseases or complex conditions. Therefore it is essential that the selected outcome measures and indicators to monitor the ERNs capture successes and failures in trying to fulfil the ERN objectives. **The goal has been to define stringent and generic indicators, which are applicable across the heterogeneity of different ERNs, and to collect data pertaining to things which *can* be changed, instead of things over which the ERNs have little or no control.**

Core Set of ERN Indicators (18)

The ERN Coordinators WG on Monitoring worked intensively from June 2017 to March 2018 in the preparation of a proposal for ERN indicators. The proposed initial set of indicators (41 indicators) was presented to the ERN Board of MS in March 2018.

The initial set of indicators was selected following a qualitative methodology. The initial agreed list proposed to monitor the ERNs, covered all seven main objectives and areas of intervention of the ERNs.

Table II: Dimensions of the proposed indicators to be assessed
• **Priority:** - clear need for the inclusion in the first set of core ERNs indicators •**Validity** –should actually measure what they are supposed to measure. •**Reliability** – the results should be the same when measured by different people in similar circumstances. •**Feasibility** – they should have the ability to obtain data when needed. •**Relevant** – they should contribute to the understanding of a phenomenon of interest.

A qualitative survey on the initial set of indicators was performed with the aim of completing and validating the initial set of ERNs indicators. Each indicator was assessed taking in account five dimensions: Priority, Validity, Reliability, Feasibility and Relevance

The final 18 indicators selection was based on the priority score and the average score of the 5 chosen dimensions mentioned above.

Application of the generic indicators to monitor ERNs

Balance is essential – the participants were in agreement that using figures for benchmarking *between* ERNs is potentially dangerous, especially those relating purely to numbers, where one can easily assume the larger the number the better the performance. **ERNs differ dramatically in size and disease scope at present.** During this process, it has been evident that the change in the patient's health as a result of ERN interventions, will suit the disease-specific monitoring of the specific ERNs. **Instead, the data collected as generic ERN indicators should be used to benchmark each ERN against *itself* over a period of time (but still with the understanding that a lack of change will not always be a negative/unavoidable thing).**

'Measuring' the latter is complex, clearly, as one can demonstrate the achievements of a Network from their creation/from the present moment: but the demonstration of 'added-value in the ERN era' entails comparisons against the care (and presumably also research etc.) provided in the pre-ERN period. Since ERNs are intended to provide the highest quality care possible, it is not ethical to 'deny' those services to patients who need them, **so one needs to look at existing statistical data within each country to attempt a comparison and assess impact**.

An important point is also to differentiate between indicators related to the ERN application forms, where each centre has to fulfil their thresholds. The assessment and monitoring of those data has been considered as one of the dimensions that would need to be completed in the upcoming months. Those data are key for understanding the performance, capacity and expertise of the members of the ERNs and of the networks as a whole and would need to be monitored and validated periodically.

When considering the ERNs patient population it is important to keep in mind that there are at least two populations to address:

- The patients that due to their complexity or need of expert advice are included in the CPMS (opening a panel) that we could name as the *ERNs CPMS population*
- The aggregated number of patients looked after by each of the HCP member of a given ERN. *The ERNs total patient population.*

While the first one (the CPMS patients) represents the individual patients and treating clinicians that would directly benefit from the expert advice of

the ERNs from a cross border perspective without the need for the patients to travel, the second one will benefit as well in an indirect way from the improvements in the knowledge, tools and expertise of the HCP that is looking after them with a national perspective.

The CPMS population represent a small percentage of the total number of patients that fall within the scope of a given ERN. They are generally those patients with rare diseases or very complex conditions where the expertise is rare that will benefit from virtual expert advice given by clinicians in different countries who pool their collective experience and expertise.

The aggregated total number of patients of an ERN (being or not referred to the virtual consultation using the CPMS) will be the backbone of the ERNs capabilities as the pooling of the data and information provided by this population of patients will feed the whole system of ERNs and make possible the generation of knowledge and new evidence for the better diagnosis and treatment of those patients. Knowledge is also being transferred to the clinician treating the patient, as they usually participate in the panel review and so directly benefit from participating in the clinical discussions with the experts on these rare or complex cases.

Any performance and outcome indicator model will need to be continually refined. For example, with time, newer outcome measures will become relevant, and some of the original measures may become redundant. This working group recommends that the quality and value of the indicators to be annually reviewed across ERNs.

The indicator specification includes suggestions of who will be responsible for collection of the data (this may be adapted to the specific ERN structures) and how frequently the data is to be collected. Each of the responsible functions will be provided with a protocol/standard operating procedure to ensure they are accurately recording the data in a comparable way.

Data collection: System to collect the data

To input and collect data, an online reporting system or an excel database should be put in place generating a series of results including customisable graphs and charts. If feasible, the monitoring system could be embedded in the already existing ERN IT platforms. According to the measures proposed, the data will be filled in at different intervals.

Who will input into the system?

Both ERN coordinators and HCPs will be responsible for providing data. How this works in practice will need to be agreed between the HCPs and the Coordinator.

Who will monitor the system?

ERN coordinators will use the system as an instrument to monitor their activities, internally manage the performance of their ERN and identify areas for improvement. It will also be a great tool to prepare for the Evaluation process every five years and guide their Self-Assessment.

Evaluation of ERNs

According to the Commission's Implementing Decision of March 2014, Article 14 clearly states that ERNs shall be periodically evaluated every five years by an evaluation body that shall draw an evaluation report for the Commission, the ERN members and the BoMs. The evaluation process is an independent requirement to the monitoring process but inevitably some of the indicators will be interlinked.

Understanding Indicators

For all identified indicators, an iterative exercise of drafting was carried out with the members of the working group. The final wording and definition of the 18th indicators was completed in November 2018.

The 18 monitoring indicators and their definitions are listed in Table V.

Table VI: basic set of 18 ERN Indicators (updated 29 July 2019)

ERN basic set of 18 Indicators	
Nº	**Indicator**
Objective 1	**To ensure that ERNs are operational**
1.1	Within an ERN, the number & percentage of Member States with full Health Care Providers as members
1.2	Number of Health Care Providers represented in the ERN
1.3	Number of affiliated partners (AP) represented in the ERN
1.4	Number of patient organisations represented in the ERNs
Objective 2	**To improve access to clinical advice, diagnosis, treatment and follow-up of patients within the ERNs**
2.1	Total number of new patients referred to the Health Care Providers participating in the ERN with the diagnosis of a disease / condition that fall within the scope of the ERN
2.2	Number of patients entered into CPMS (total volume)
Objective 3	**To optimise patient outcomes by combining healthcare professionals' skills & resources used**
3.1	Number of patients entered into CPMS and reviewed by the ERN (a panel case review which leads to an outcome report)
3.2	Time taken to provide multidisciplinary clinical advice - non-urgent cases: days (median) between referral to ERN and multidisciplinary clinical advice
Objective 4	**To increase capacity of professionals to recognize and manage cases of rare and complex conditions and diseases within the scope of the ERN**
4.1	Number of educational webinars/videos aimed at healthcare professionals delivered by the coordination or HCPs members of the ERN
4.2	Number of formal educational activities (i.e. those accruing higher educational credits) aimed at healthcare professionals organised by the ERN
Objective 5	**To reinforce clinical research in the field of rare and complex conditions and diseases by collecting data and carrying out research activities**
5.1	Number of Clinical Trials or Observational prospective studies (with > 1 Member State and Health Care Provider within the ERN)
5.2	Number of accepted peer-reviewed publications in scientific journals regarding disease-groups within the ERN and which acknowledge the ERN
Objective 6	**To ensure that patients referred to ERNs have equal access to high and quality healthcare**

	services	
6.1	Cumulative number of Clinical Practice Guidelines and other types of Clinical Decision Making Tools and the number of new ones, **adopted** for diseases within the scope of the ERN	
6.2.	This indicator is split into two sub indicators: 6.2.a Cumulative number of Clinical Practice Guidelines and the number of the new ones **written** by the ERN 6.2.b Cumulative number of other types of Clinical Decision Making Tools (clinical consensus statements or consensus recommendations) and the number of new ones, written by the ERN for diseases within the scope of the ERN	
Objective 7	**To guarantee that knowledge is spread outside the ERN so that more people can benefit from the ERN activities**	
7.1	Number of congresses/ conferences/ meetings at which the ERN activities and results were presented	
7.2	Number of individual ERN website hits	
Objective 8	**Complex and long-term indicators which need further development**	
8.1	Level of patient satisfaction	*To be developed*
8.3	Health Care Provider Compliance to Clinical Guidelines	*To be developed*

Table V: Basic set of 18 ERN Indicators including definitions, clarifications and examples (updated August 2019)

ERN basic set of 18 Indicators		
Nº	**Indicator**	**Definition**
Obj 1	**To ensure that ERNs are operational**	
1.1	Within an ERN, the number & percentage of Member States with full Health Care Providers as members	*Within a particular ERN, the total number of Member States with at least one full Health Care Provider member within that ERN, also shown as a percentage of the total number of Member States with the EEA covered by Directive 24/201 (currently 29).*
1.2	Number of Health Care Providers represented in the ERN	*The total number of full Health Care Providers within the ERN. (Also to consider for the next yearly collection – 2019- the average or median number by ERNs and the range.)*
1.3	Number of affiliated partners (AP) represented in the ERN	*The total number of affiliated partners (APs) within the ERN. Idem as above*
1.4	Number of patient organisations represented in the ERNs	*The total number of patient associations represented by one or more persons actively involved in the ERN.* *Patients may work within an ERN in many different ways to capture their voices and their needs.* *To have a clearer idea of the participation of patients in the ERN, the following types of involvement should be, where possible, counted and reported in the comments box of the monitoring data collection IT tool.* *Examples of the types of active participation in an ERN network (and therefore should be counted towards the total number) are:* *Number of Patient associations represented:* *1) as voting members of the Board of the Network (please count the patient associations represented that are entitled to vote in the decision-making bodies governing the ERN);* *2) as Leader (or co-Leader) of specific activities of the ERN project (please count the patient associations represented and involved in working groups, work packages, tasks, etc. as Leader or co-Leader);* *3) as members of the panel involved in the production of clinical practice guidelines (please count the number of patient association represented during the process of creation of new clinical practice guidelines*

		or adaptation - both as adaptation to the countries and adaptation in lay versions - of existing clinical practice guidelines); 4) *as co-designer of activities related to the Network project (please count the number of patient associations represented and involved in the main activities of the ERN, such as co-design of surveys, training and education, website contents, dissemination materials, etc.);* 5) *that are actively involved in translation of ERN documents, evaluation of patient information, and other ERN documents, including proposing changes (to ensure they are suitable for patients or parents)* *Participation of patient associations in other type of meetings directly related with the work of a given network (ePAGs meeting, ectorial or thematic patient associations meetings, etc.) should also be counted.* ***Clarifications and examples:*** • *To clarify that this indicator does not aim to count the number of meetings, nor the type of meeting in which patient representatives are participating.* • *Such active involvement would include their participation in advisory groups, committees, and any other bodies within the organization of the network.* • *This participation would normally be reflected in the membership and their attendance at the meetings (physical and virtual) of that body.* • *In the case of umbrella organizations (for example, EURORDIS) please count each of the umbrella organizations once and count only once the other individual associations represented (whether that be European, national or regional). For example: a patient representative that belongs both to EURORDIS and to a national association of disease X will be counted as 2 patients associations.* • *With regards to umbrella organisations, please indicate in the comment box the name of each umbrella organisation represented and the type of coverage they have (e.g. national, European or multi-disease coverage).* • *Patients associations represented by more than one person or in different advisory groups or committees or any other bodies of the ERN will be counted just once.*
Obj 2	**To improve access to clinical advice, diagnosis, treatment and follow-up of patients within the ERNs**	

2.1	Total number of new patients referred to the Health Care Providers participating in the ERN with the diagnosis of a disease / condition that fall within the scope of the ERN	*The total number of new patients attending the ERNs' Health Care Providers for the first time, whatever their age, within the specified timeframe, including visits to outpatient's clinics, hospital discharges and emergencies, coming from both national and international referrals whose disease/condition falls within the codes listed*[1]*.* ***Clarifications and examples:*** • *New patients are those that have attended or been referred to the center, within the specified timeframe and having a certified diagnosis of a rare disease.* • *Patients are still considered to be new patients where they have attended or been referred to the centre previously but not under a certified rare disease diagnosis code.* • *Patients that are still within the timeframe/procedures needed for the diagnosis will not be included in the counting.* • *In a number of instances, the number of new patients seen each year for some rare diseases will be very low. However, it is the intention of this data collection process to establish a baseline for each healthcare provider, rather than comparing numbers between ERNs.* • *There are important differences depending on the ERNs on the type of contact with the Hospital. Some ERNs are mainly having outpatient visits while others are mainly focusing on hospital discharges recurrent patients shall be counted once. These clarifications should be noted, as far as is possible, in the comments box of the monitoring data collection IT tool.*
2.2	Number of patients entered into CPMS (total volume)	*The total number of unique patients entered into CPMS within the specified timeframe for that ERN.* ***Clarifications and examples*** • *This measure aims to capture the total number of patients referred to CPMS, regardless of whether a panel has been created or advice has been provided to the treating clinician.* • *Only real patients cases should be counted and the correction of the data should be done accordingly – CPMS must only be used for real patients. Any testing of the system must be done on the CPMS training environment which functions exactly the*

1The disease should be preferably confirmed at the moment of the data inclusion by using, in principle, the same codes as those specified in the ERNs disease-area breakdowns. Depending on the particularities of some diseases, patients still under diagnosis process could be included as referred patients.

		same as the real system.
Objective 3	**To optimise patient outcomes by combining healthcare professionals' skills & resources used**	
3.1	Number of patients entered into CPMS and reviewed by the ERN (a panel case review which leads to an outcome report)	*The total number of patients who have been entered into CPMS within the specified timeframe and whose case is subsequently reviewed by a panel that consists of at least three experts or for bilateral consultation between two experts and for which an outcome report is produced.* ***Clarifications and examples*** • *CPMS must only be used for real patients. Any testing of the system must be done on the CPMS training environment that functions the same as the real system.* • *If your ERN is not using CPMS please report this in the comment box and report the number of cases where expert advice has been given to the treating healthcare professional. In such circumstances, please ensure that only ERN activity that mirrors the CPMS process is counted.*
3.2	Time taken to provide multidisciplinary clinical advice - non-urgent cases: days (median) between referral to ERN and multidisciplinary clinical advice	*The days (expressed by the median) for the time period specified between the date of start of the panel in CPMS*[2] *and the date of issue of multidisciplinary clinical advice (outcome report)*[3] *independently of the closure of the panel from the created panel for that same patient, where at least three experts have participated or for bilateral consultation between two experts.* ***Clarifications and examples*** • This figure is generated by CPMS for those ERNs using it. It does not need to be calculated by ERNs. For ERNs not using CPMS, please add this data in the comment box. • Example: The case of a patient who seeks a second opinion, advice on highly specialised surgery or confirmation of a suspected diagnosis: The medical information is uploaded to CPMS (the clock starts), a panel opens, the advice is agreed upon and CPMS produces an outcome report which could give the second opinion, confirm or not the diagnosis and/or

2 The time point measured automatically by CPMS will be the time from the start of the panel in CPMS until the production of the outcome report.

3 CPMS outcome report created and sent to the treating clinician i.e. the clinician who is responsible for treating the patient in the Member State where the patient lives.

		recommend that the surgery is carried out in a healthcare provider with expertise in this type of surgery. The clock stops when the outcome report is produced by CPMS or for those ERNs not using CPMS, when the advice is sent to the treating clinician. It is recommended as best practice that the treating clinician shares the outcome report with the patient as the basis of their discussions. This way the patient can see the added value of the ERN. The ERN may or may not decide to close the panel at this point as further information about the follow up of the patient may be included.

Objective 4	**To increase capacity of professionals to recognize and manage cases of rare and complex conditions and diseases within the scope of the ERN**	
4.1	Number of educational webinars/videos aimed at healthcare professionals delivered by the coordination or HCPs members of the ERN	*The total number of educational webinars[4] and/or shorter videos aimed at healthcare professionals and/or patients created and delivered on an appropriate platform by the ERN coordination team or HCPs members of the ERN within the specified time period.* ***Clarifications and examples*** • *The number of unique webinars delivered by an ERN within the specified timeframe that are publically available (e.g. on websites) should be counted.* • *If a webinar with the same content is delivered 3 times in one year, this should be counted as 1.* • *Webinars should feature to ERN logo.* • *Only count new editions, not repetitions.*
4.2	Number of formal educational activities (i.e. those accruing higher educational credits) aimed at healthcare professionals organised by the ERN	*The total number of formal educational activities (i.e. those accruing higher educational credits) certified by a formal educational body.* ***Clarifications and examples*** • *The body shall have recognized capacity (at regional, national, EU, or International level) to issue educational credits.* • *The credits should be aimed at healthcare professionals members or non-members of the Networks organised (including co-organisation or with important contribution) by the coordinating centre of the ERN or by one or more HCPs of the*

[4] A webinar is a seminar conducted over the internet.

		ERN. • *The activity should acknowledge the ERN participation (including the logo of the ERN) within the specified time period.* • ***Example (ReCONNET experience):*** *An ERN highly involved in the scientific organizing committee in a CME course of one of the diseases covered by the network with a relevant contribution of their HCP as trainers.* *A request of a formal endorsement was submitted to the decision-making body of the ERN that approved the request enabling the organizer to acknowledge the ERN and to add the ERN logo to the materials of the course.* *Only after ensuring that all the requested criteria were met, the network included this course as formal education activity of the ERN.*

Objective 5	**To reinforce clinical research in the field of rare and complex conditions and diseases by collecting data and carrying out research activities**	
5.1	Number of Clinical Trials or Observational prospective studies (with > 1 Member State and Health Care Provider within the ERN)	*The total number of ongoing Clinical Trials or Observational Prospective Studies (including both academic and Industry driven studies) within the specified time period that involve at least two Health Care Providers from two different Member States within the ERN, acknowledging the ERN.* ***Clarifications and examples*** • *This indicator is asking for the number of trials or observational prospective studies that* *a) involve HCPs within an ERN, and* *b) includes an acknowledgement of the ERN.* • *Ongoing trials may be counted, but ERN involvement must have been acknowledged at that point.* • *Where ERN involvement has been confirmed, the trial should be counted in the period in which the trial started.* • *These qualifying criteria can be presented together or in different documents.* • *Providing a reference for each study in the comment box could be very useful: https://clinicaltrials.gov/ct2/home https://www.crd.york.ac.uk/prospero* • *Transversal studies such as genotype/phenotype correlation studies such can be counted as clinical*

		trials (as clinical data are used on a group of patients within the ERN) as long as they acknowledge the ERN participation (including logo of the ERN) within the specified time period. • *The clarification of possibilities and limits regarding the cooperation with Industry is not a concluded process. The statement of ERN Board of Member States has been recently updated – 25th June 2019. This is impacting on the involvement of HCPs as ERN members in Industry driven studies, because ERNs do not have a clear view about how this kind of collaboration can be run – at the moment.* • *For this reason, many HCPs have not acknowledged the ERN in the study, and have therefore not counted Industry driven studies in the collection of data.* ***Examples which should be counted**:* *1) See clinicaltrials.gov where the study clearly acknowledges an ERN (ERN-NMD) in the study description:* *https://clinicaltrials.gov/ct2/show/NCT03857880?id=NCT02971683+OR+NCT03189875+OR+NCT02419365+OR+NCT03857880&rank=1&load=cart* *2) See clinicaltrials.gov where the study involves more than two HCPs of ERN (ERN ReCONNET) but there is no clear acknowledgment of ERN; in this case a document with a clear statement of participation of the ERN will be made available as annex (see Table VI):* *https://clinicaltrials.gov/ct2/show/study/NCT03189875?id=NCT02971683+OR+NCT03189875+OR+NCT02419365+OR+NCT03857880&rank=2&show_locs=Y&load=cart#locn*
5.2	Number of accepted peer-reviewed publications in scientific journals regarding disease-groups within the ERN and which acknowledge the ERN	*The total number of accepted peer-review publications in scientific journals regarding disease-groups within the ERN and within the specified time period.* *Publications should be PubMed accredited scientific journals and involve as major contributors at least two Health Care Providers from two different Member States within the ERN, and which acknowledge the ERN (see acknowledgments examples in table VI).* ***Clarifications and examples*** • *If publications reference the ERN in the affiliations or acknowledgements in a way that is*

		different from acknowledgments examples in Table VI (e.g. they were published before the definitions were available), they can be included • *For counting purposes, ERNs should only be counting those publications that fit number 2 in Table VI at the end of this document.* • *Other publications could be listed in the comment box.* • *A clinician in a HCP could be very active in producing publications but they could have nothing to do with the ERN or its activities.* • *The figure captured here should be clearly linked to the ERN and its activities.* • *To add in all the cases of acknowledgement the definitions listed in Table VI at the end of this document.* ***Example** (ReCONNET)* • *12 peer-reviewed publications about the results of ERN ReCONNET activities on clinical practice guidelines carried out during the first 18 months had been published at the end of 2018.* • *These publications are included in the supplement "ERN ReCONNET Supplement on the state of the art on CPGs in rCTDs". It was officially published after a peer-review process of each single article. The Supplement is already available in the RMD Open website (https://rmdopen.bmj.com/content/4/Suppl_1).* • *Each publication has a different Pubmed ID code.* • *After consulting the Communication experts within the ERN policy team within the EC, each publication reports the acknowledgment statement regarding the EU funding and the n. 24 ERNs.* • *Moreover, the ERN logo is included – in each publication.*
Objective 6	**To ensure that patients referred to ERNs have equal access to high and quality healthcare services**	
6.1	Cumulative number of Clinical Practice Guidelines and other types of Clinical Decision Making Tools	*The cumulative number of Clinical Practice Guidelines (CPG)[5] and other types of Clinical Decision Making Tools[6]*

[5] Clinical practice guidelines are statements that include recommendations intended to optimize patient care that are informed by a systematic review of evidence and an assessment of the benefits and harms of alternative care options. Clinical Practice Guidelines We Can Trust. Robin Graham, Michelle Mancher, Dianne Miller Wolman, Sheldon Greenfield, and Earl Steinberg, Editors; Committee on Standards for Developing Trustworthy Clinical Practice Guidelines; Institute of Medicine 2011).

	and the number of new ones, **adopted** for diseases within the scope of the ERN	*such as clinical consensus recommendations for disease areas within the scope of the ERN that have been adopted during the specified timeframe, formally agreed by the ERN Board, are publically available (eg on website) and use the ERN logo.* ***Clarifications and examples*** • *"The ERN has adopted the CPG or Clinical Decision Making Tools" means that the tools are publically available and all the HCPs within a network are following the guidance.* • *The adaptation of the CPGs already existing appears a very crucial added value of the ERNs, since the adaptation may increase the application of CPGs by healthcare professionals. The adaptation of CPGs can be done by means of the ADAPTE methodology that guarantees the production of defined priorities to be followed across Member States.* • *The adoption of CPGs within an ERN could be defined, for example by means of an official endorsement of the Board of the ERN.*
6.2.	This indicator is split into two sub indicators: 6.2.a Cumulative number of Clinical Practice Guidelines and the number of the new ones **written** by the ERN 6.2.b Cumulative number of other types of Clinical Decision Making Tools (clinical consensus statements or consensus recommendations) and the number of new ones, written by the ERN for diseases within the scope of the ERN	• *The cumulative number of Clinical Practice Guidelines (CPG) or Clinical Decision Support Tools (CDST: clinical consensus statements or consensus recommendations) developed by the ERN, shall involve at least two Health Care Providers from two different Member States within the ERN, acknowledging the ERN, for diseases within the scope of the ERN where no guidelines existed previously, according to evidence based recognized methodology.* • *The new CPGs or CDST should be developed by the ERN during the time frame measured.* ***Clarifications and examples*** *As above* • *ERNs have very different scenarios with reference to the number of diseases covered and also to the number of already existing CPGs.* • *It is important to underline that for some diseases, many CPGs are already available, for other rare diseases there are no CPGs available at the moment as there is insufficient evidence to produce new CPGs.*

[6] A clinical consensus statement is the end product developed by an independent panel of (at least 3) subject matter experts convened specifically to perform a systematic review of the available literature, for the purpose of understanding a clinically relevant issue or surgical procedure. It offers specific recommendations on a topic. Compared to Clinical Practice Guidelines and Clinical Practice Recommendations, Clinical Consensus Statements undergo a less rigorous peer review process.

		• *The differentiation between evidence based Clinical Practice Guidelines (CPG) and other Clinical Decision Support Tools (CDST) based on consensus techniques (mainly expert or consensus recommendations) is important when identifying the elements to count. Currently the main criteria to distinguish CPG from Consensus recommendations shall be the standard definition of CPG* • *The Clinical Practice Guidelines (CPG) and other Clinical Decision Support Tools (CDST) based on consensus techniques to be counted shall be those written within the measured timeframe by the ERN (eg agreed by the ERN Board), not when they are published.* • *Measuring only the new CPGs produced by the ERN is probably not sufficient to monitor the improvement of the access of patients to high and quality health care services. CDST production shall be consider crucial* • *In many cases the role of ERNs would be to collect the evidence that will represent the needed base for the creation of CPGs. This will be done also through the ERN Registries.* • *Another important element that should be considered in measuring the equal access to high and quality health care is the adaptation of CPGs in the different Member States.* • ***Example:** for those diseases that already have published CPGs, ReCONNET is performing an adaptation of the guidelines in the different contexts by means of the ADAPTE methodology.* • *Additional elements could be considered in the future as sub-indicators for 6.2 in order to capture relevant activities of ERNs related to the improvement and harmonization of care across Europe, not limiting to the creation of new CPGs, but also including adaptation, generation of new evidence, new clinical tools for monitoring the diseases, etc.*
Objective 7	**To guarantee that knowledge is spread outside the ERN so that more people can benefit from the ERN activities**	
7.1	Number of congresses/ conferences/ meetings at which the ERN activities and results were presented	*Within the specified time period, the total number of congresses/ conferences/ meetings at which the ERN activities and results were presented via a dedicated slot in the programme/agenda, acknowledging the Network and including the ERN logo.*

		Clarifications and examples • *The aim of this indicator is to capture the dissemination activities of the ERNs.* • *Please count the presentations made by your members which contain information about the activities of the ERN.* • *The presentation must feature the ERN logo.* • *Please do not count presentations where the ERN is just mentioned.* • *The ERN and its activities should be the focus of the presentation.*
7.2	Number of individual ERN website hits	*The total number of page views including both the homepage of the website and the "child" pages.* ***Clarifications and examples*** • *There are different tools available that could help avoiding to count the machine-visits and include only actual page visits* ***Example:*** *Please use the google analytics tool for the counting, where "page visits" is a specified variable: https://analytics.google.com/analytics/web/*

Objective 8	**Complex and long-term indicators which need further development**	
8.1	Level of patient satisfaction	*To be developed*
8.3	Health Care Provider Compliance to Clinical Guidelines	*To be developed*

Table VI: Acknowledgement to be used in publications, clinical trials, educational activities or guidelines

Acknowledgement <ERN to be specified>	Situation
1. " The two (or more) of the/several author(s) of this publication is/are (a) member(s) of the European Reference Network for … - Project ID No 739543."	A general option that members can use regardless of there being 2 or more HCPS involved. This gives attention to the existence of ERN without it acknowledging any direct input from it.
2. "This work is generated within the European Reference Network for …	An option that an HCP can choose to add if the work has come into being by the work carried out by at least 2 or more ERN members working within the structure of the network (WP/SNW)
3. "This study/project/publication/Guidelines/survey* has been supported by …. , which is partly co-funded by the European Union within the framework of the Third Health Programme "ERN-2016 - Framework Partnership Agreement 2017-2021." ***choose appropriate wording**	If funding is allocated to a publication/ project/etc*.