Continuous Monitoring of ERNs

ERN Continuous Monitoring and Quality Improvement System (ERN CMQS)

Set of ERN core indicators (18)

Version v.1

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

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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
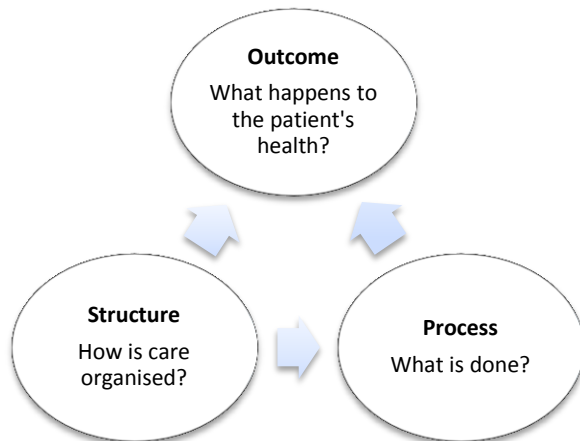
Why do we need a continuous monitoring system for ERNs

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

Intervention area: General organisation and coordination
- Objective 1: To ensure that ERNs are operational and successfully carry out their organisational activities

Intervention area: Patient Care
- Objective 2: To improve access to clinical advice, diagnosis, treatment and follow-up of patients within the ERNs

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

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

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

- **Intervention area: Clinical guidelines**
- Objective 6: To ensure that all patients referred to ERNs have access to high quality healthcare services

Intervention area: Communication and dissemination within the scope of the ERN activities
- Objective 7: To guarantee that knowledge 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 V: basic set of 18 ERN Indicators

Nº	Indicator	Definition
ERN basic set of 18 Indicators		
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.*
1.3	Number of affiliated partners (AP) represented in the ERN	*The total number of affiliated partners (APs) within the ERN.*
1.4	Number of patient organisations represented in the ERN meetings	*The total number of recognised patient organisations [1]represented within ERN meetings as agreed by the ERN Board.*
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 Health Care Providers with diseases / conditions that fall within the scope of the ERN	*The total number of new patients referred to the ERNs' Health Care Providers within the specified timeframe whose disease/condition falls within the codes listed[2].*
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.*
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)	*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.*
3.2	Delay 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 enrolment of a new patient to CPMS[3] and the date of issue of multidisciplinary clinical advice [4](i.e. panel closure) from the created panel for that same patient, where at least three experts have participated or for bilateral consultation between two experts.*
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 aimed at healthcare professionals delivered by the ERN	*The total number of educational webinars[5] aimed at healthcare professionals created and delivered on an*

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[1] Patient organisations are defined as not-for profit organisations which are patient focused, and whereby patients and/or carers represent a majority of members in governing bodies. Each ERN Board will define the type of and the number of meetings where Patients Representatives are expected to participate.

[2] The 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.

[3] Creation of a patient record in CPMS and invitation of experts to a panel to review this case

[4] CPMS outcome report created and sent to the treating clinician ie the clinician who is responsible for treating the patient in the Member State where the patient lives.

[5] Webinar is a seminar conducted over the internet

		appropriate platform by the ERN within the specified time period.
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) aimed at healthcare professionals organised by the ERN within the specified time period.*
Objec tive 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 Clinical Trials or Observational Prospective Studies within the specified time period that involve at least two Health Care Providers of two different Member States within the ERN.*
5.2	Number of accepted peer-reviewed publications in scientific journals regarding disease-groups within the ERN and which name 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 of two different Member States within the ERN, and which specifically name the ERN.*
Objec tive 6	**To ensure that patients referred to ERNs have equal access to high and quality healthcare services**	
6.1	Number of Clinical Practice Guidelines adopted for diseases within the scope of the ERN	*The total number of Clinical Practice Guidelines (CPG)[7] adopted for diseases within the scope of the ERN, according to evidence based recognised methodology.*
6.2	Number of new Clinical Practice Guidelines written by the ERN	*The total number of new Clinical Practice Guidelines (CPG)[6] developed by the ERN for diseases within the scope of the ERN where no guidelines existed previously, according to evidence based recognised methodology.*
Objec tive 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.*
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.*
Objec tive 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*

[6] Clinical practice guidelines (CPGs) are statements that include recommendations intended to optimise patient care that are informed by a systematic review of evidence and an assessment of the benefit and harms of alternative care options (IOM).