Continuous Monitoring of ERNs

Version 2

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Non-paper

Disclaimer: This paper should be solely regarded as a basis for discussion and cannot in any circumstances be regarded as the official position of the Commission and its services

Continuous Monitoring of ERNs

Introduction

The ERNs legal framework sets up the objectives, principles, 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 identify the outcomes of the ERNs and ultimately to learn from the experience identifying success and potential pitfalls and to demonstrate the value of the ERNs.

The process to set up such a monitoring and information system implies 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.

This is a preliminary document to guide and facilitate an initial discussion with the ERN coordinators.

Following this initial proposal, the next step is to discuss and agree on a methodological approach for building the indicators and endpoints (in the case of the outcomes) and at a later stage, validate a functional monitoring system.

To enhance and complete a robust ERN monitoring and assessment system it is important to look into 4 dimensions:

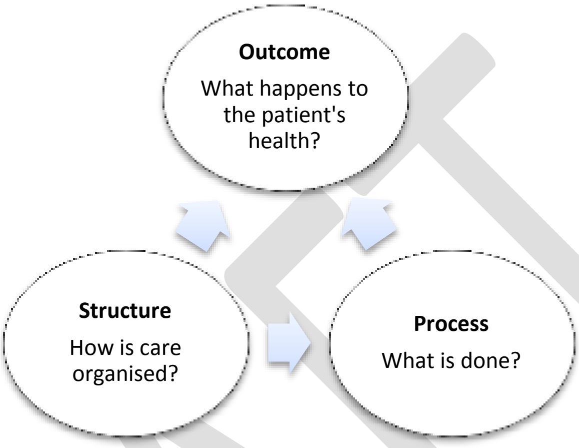
1. Development of a continuous monitoring system of the ERN activities
2. Periodical self-assessment and reporting of the ERNs and HCPs (similar to the Assessment performed at the initial stage);
3. Stronger involvement of Member States in the assessment of their national HCPs wishing to participate in, or participating already in related ERNs
4. Further assessment by the IAB of HCP not assessed in the initial process

Several actors will be involved in the above dimensions namely the ERN coordinators, the ERN Board of Member States (BoMs) and the European Commission (DG SANTE).

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.

Understandably, each ERN will also have specific indicators based on some technical aspects specific to each ERN and on the conditions that they each address. Individual indicators will need to be discussed internally within each ERN and with the ERN coordinators in order to find further commonalities.

Figure 1: Donabedian model



Why do we need a continuous monitoring system for ERNs

The ERNs lifecycle follows the PDSA model: Plan, Do, Study, Act. Following an initial Plan resulting in the implementation of the ERNs (Do), 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.

A monitoring system for ERNs would:

- 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 are producing something useful for the 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 when objectives aren't met

ERN goals - Identifying common objectives

Legal framework for ERNs

The Directive 2011/24/EU is intended to provide 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

In order to design a monitoring system that answers to the general objectives of 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.

Intervention area: General organisation and coordination

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

Intervention area: Patient Care

- Objective 2: To improve access to clinical advise, 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 and complex conditions and diseases within the scope of the ERN

Intervention area: Contribution to research and innovation

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

The main aim of the exercise of definition of ERN indicators should be to better reflect the indicators that are most meaningful for the ERNs environment and to be able to provide accurate reporting to healthcare authorities, patients, health care providers and clinical and research experts .

Any performance and outcome indicators model will need to be continually refined and expanded. A stable set of key performance indicators can be used to identify opportunities for improvement and will help ensure cohesion across the EU health care system.

The framework below presents a visual summary of the ERN objectives and indicators for monitoring ERN performance. An important point is to differentiate between indicators related to the ERN application forms where each centre has to fulfil their thresholds and indicators that have to do with the work within the network.

UPDATE – To produce the second version of this table of proposed common Indicators for all ERNs to collect, RD-ACTION organised a dedicated workshop, jointly with DG SANTE, which took place in Newcastle UK on 1st -2nd June.

Table 1: Indicators and their characterization based on the identified ERN common objectives

There are some important principles to consider in agreeing the contents of the table below:

Many of these proposed Indicators were based upon what the ERNs themselves proposed to collect and monitor – i.e. they came from the Coordinators! Even if the Indicators are challenging to agree in some –or all!- areas (i.e. objectives, as stipulated in the first column), each of these relates to a mandatory task of the Networks and we must therefore persevere to find granularity and a reasonable compromise: one cannot simply jettison 'research' for instance. We should look at external sources to support, where relevant (e.g. NIHR is very keen on research metrics, so could be a good reference)

What are we measuring? We need to demonstrate that the networks are functioning, but simultaneously –and particularly important, in terms of longevity- are adding value compares to what exists. 'Measuring' the latter is far more complex, clearly, as one can demonstrate the achievements of a Network from their creation/from the present moment, via data collected 'in a vacuum': 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 to existing statistical data within each country to attempt a comparison and assess impact.

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. Instead, the data collected 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)

Scope of these Indicators - It is important to clarify whether we are seeking to collect data pertaining to things which *can* be changed, or things over which the ERNs have little or no control

Understanding the changes made to the Table:

- Once the WG advances further in its discussions, it will need to agree the frequency at which the Indicator data is reported (will all data be collected annually?) and also discuss WHO will collect the data for each row, and how…
- The table has been significantly amended, largely through annotation, to reflect the comments provided by workshop participants (both in the group work and in plenary).
- The 'Comments' column of the table below has been expanded significantly. This is designed to act as a record of discussion points, to support the necessary decision-making to progress with the selection and finalisation process.
- The *Italic text* in the Comments section represents the original text, as per the first draft – everything else has been added after the Newcastle workshop.
- An additional column has been added to the table, on the right-hand side. This was intended to illustrate one of the following:
    - Concrete revisions proposed by the workshop groups, where they had been able to reach some degree of consensus on rewording OR where they had opted to abandon an indicator entirely (in the case of the latter, this has been illustrated in column 2 using text ~~strike-through~~)
    - Proposals to help the WG finalise the wording of the Indicator in question
    - Where the wording/qualification appears very open, and broadly different interpretations are possible, options have often been proposed to support the next steps (in terms of *what* to include, how to *phrase* the Indicator, and what needs to be defined/qualified for the Indicator to be used)

ERN Specific objective	**Indicator name (Original Non-Paper version)**	**Indicator type (structure, process, outcome)**	**Comments from the Groups and Plenary**	**Revised Indicator Name (proposed)/action required**
To ensure that ERNs are operational[1] ~~and successfully carry out their activities~~	**Number of MS represented in the network**	Structure	Groups were divided here, as to whether to keep this Indicator: • On the one hand, the info is easily collected, which is a bonus, but is there an added value to collecting this information? For one thing, it is already known from the application (although with future membership changes, it may change). • Others argued that this Indicator *is* important as it's a way to maintain 'pressure' on the MS to participate in all ERNs (i.e. we should all be aiming for 28) • A key point was raised here, namely, **should these Indicators be capturing things which the ERNs cannot control?** The endorsement of MS HCPs is beyond the power of an ERN to control – the decision on whether to participate or not lies with the MS, thus is it appropriate to capture here? When opened up for Gp discussions, some agreed that this was more relevant for a sort of 'state of play' exercise, with such data perhaps being collected by other bodies such as the BoMS. It is important to collect this information as it will help ensure geographical coverage for benefit of RD patients and	One Gp proposed to abandon this one, but opinion was divided (as we noted in the plenary too). If it is retained: • Need to agree if the *ERNs* should collect this themselves, or another group (such as the BoMS) • Need to agree scope – if APs are included, and we count 'representation' as either full membership or a form of 'affiliation', title should be adapted to something like

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[1] The Gp felt that 'successful carrying out of activities' would encompass more than the 'operational' activities specified here – they agreed to rephrase this Objective therefore, to be more focused on the 'nuts and bolts' of ERN operationalisation and less on success.

			supports comparison across nations/ERNs but should not necessarily be viewed as an indicator of success. If it *is* maintained, we need to clarify if 'affiliated' partners count here too, and if so, whether a single total should be provided (i.e. a MS is counted if they have either a full member or an 'Affiliated' member in a given ERN)	**'Total number of MS represented – as full or else formally 'affiliated' members- in the ERN**
	Number of Working Groups set up	Structure	Numbers alone may not be so relevant – again, it may imply that the higher the number, the better, when in reality, most ERNs will set up all of their internal WGs in the first year and the number will not change. Some participants favoured *keeping* this one, but comparing it more closely against the objectives each ERN set out for itself in the 1-year and 5-year workplans: so measuring % set-up against targets. Another Gp proposed that one would need to define 'Working Groups' here as WGs initiated under each ERN (to distinguish them from other WGs e.g. those under the ERN CG). They proposed splitting this to: 1. No. of disease-specific working groups set up under each ERN 2. No. of transversal working groups set up under each ERN (assume this would include things like NGS groups, Guidelines groups etc)	**Need to choose from one of these options:** - Keep it as a single numerical Indicator - Divide it into two more specific Indicators, as proposed by the second Gp, collecting each numerically - Amend it to record **'percentage of Internal ERN Working Groups established, relative to the ambitions of the workplan'**
	Number of ERN members participating in ERN meetings (discard?)	Process	*To further discuss on the type of meetings (Board, Advisory, Working group meetings).* One Gp felt that this Indicator was not meaningful and should	Either abandon or Consider changing to: **"Proportion/percentage**

			be abandoned – it will vary naturally between ERNs, purely because of the size difference. If it is retained, it should perhaps be captured as a percentage or similar. The second Gp preferred to keep it but to specify that it referred "organisational meetings"	**of total HCPs members participating in organisational meetings"**
	~~**Number of MS participating in ERN meetings**~~	Process	As per the previous row, the Gp felt that this was not meaningful and should be abandoned	N/A: Gp proposed to abandon this one
	Number of meetings (Board, Working Groups…)	Process	*Discuss whether we can agree on a common type of meetings* Both Gps were in favour of keeping this Indicator. One Gp was content with broad definition (i.e not breaking it down), adding again though that this is more useful as a descriptive indicator and not as an indicator of success The second group did not really have a chance to specify the types of meeting, but a reasonable proposal might be 'Number of 'Operational' meetings of the ERN, including Board, Executive/Steering Committee (or equivalent) and Internal WG meetings.' We could either combine all these categories into one, or propose a separate Indictor for each (NB, one would not count patient-case-focused meetings, such as conducted via the CPMS)	'**Number of 'Operational' meetings of the ERN, including Board, Executive/Steering Committee (or equivalent) and Internal WG meetings.**' (VH – this is only my own suggestion)
	Number and type of stakeholder organisations involved in the advisory bodies **- Patient organisations** **- Professional associations**	Structure	*Stakeholders are defined as organisations that are not members of the ERN* Although one Gp agreed that including external stakeholders is important, there was a concern that these things cannot always be controlled by the ERNs – if you have few POs interested, it will be difficult to increase this number.	Either abandon (one Gp was in favour of abandoning this Indicator[3]) or else - Agree to count together or 3 times, one for each

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[3] VH I disagree with this, actually – we need to record the wider engagement of the ERN somehow, and this is fairly open with respect to what a 'advisory group' is, which is good. As long as one appreciates that the numbers may not rise after the 1st year or so –at least for the last 2 types of stakeholder- this should be fine

	- Scientific associations		If we do keep this Indicator, we will need to decide whether to count the number of *each* of the three types of 'stakeholder organisations separately (e.g. how many POs, how many Professional Associations etc') or count the combined total (the former would be more meaningful surely). Either way, one would need to define 'involved': an example could be (VH) 'either joining an advisory body as a formal member OR participating to at least one call/meeting of an advisory body over the course of a year' The second Gp proposed splitting the indicator into two yes/no questions: [2] 1) "are patients involved in activities of ERN" 2) "are scientific/professional" associations involved in activities of ERN"	stakeholder group; AND • Agree a definition of 'involved' as per my example left
	NEW INDICATOR PROPOSED	Structure	One Gp felt that patient involvement in the ERN was not really captured very specifically and warranted its own Indicator. This could focus on ePAG involvement, as proposed here: however, one could go further, and include no. of instances of patient participation to operational meetings above (this will probably encourage patient representation in these meetings, including internal WGs – desirable, surely?)	**'Number of ePAG members'**

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[2] VH -However, the information will then be fairly meaningless for an ERN seeking to track its performance and engagement over time, and to demonstrate impact. I assume that we expect all ERNs to engage somewhat with both categories, and thus numbers -although not given undue weight- are needed, to show concrete impact.

To improve access to clinical ~~advise~~ advice, diagnosis, treatment and follow-up of patients within the ERNs[4]	**Average time between referral to ERN and multidisciplinary clinical advice.**	Process	*To discuss on common definition of clinical advice* The most obvious issue here is the need to define 'referral'. The Groups were unanimous in this, and it was echoed in the plenary discussion. The most logical way to distinguish between a patient of the ERN and a patient of the HCP more broadly, is to define 'referral to the ERN' as the act of creating a case in the CPMS.[5] One Gp wished to replace "clinical advice" with "clinical consultation". This seems preferable, as 'advice' opens up more questions on definitions, regarding what the patient might receive. The Gp therefore proposed changing this Indicator to "average time from entry of patient into CPMS to beginning of clinical consultation in ERN".[6] It is important to agree what exactly we wish this Indicator to capture – is it the time from a case referral via the CPMS to the end of the multidisciplinary review? If so, do we define the latter as the time when a report is generated and 'signed-off'?	Revised wording might be one of these: **"Average time from entry of patient into CPMS to beginning of clinical consultation in ERN"** (proposed by one group) OR **'Average time between referral to ERN (i.e. creation of a case form[7] via the CPMS and invitation of experts to review this case) and the receipt of multidisciplinary clinical advice (i.e. return of a CPMS encounter report to referring clinician)**

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[4] There was strong agreement that this section, whilst essential, is problematic in its current form – the best way forward is to agree on certain definitions, the options for which I have attempted to present in the table. The groups also clearly ran out of discussion time for some of these, but noted that Indicators such as Survival, Mortality and Morbidity need far more discussion (and some of the wording will really be difficult to agree in a common table, as each ERN typically – and naturally- has a very focused view.

[5] This assumes however that the CPMS is used only for cases requiring consultation from at least one other expert in the ERN. If cases can be opened for internal use -i.e. if an HCP plans and is able to use the CPMS for more of an internal or personal 'case management' tool, this definition will need more finetuning.

[6] The problem here is, how do you define beginning of the consultation? When the first expert contributes? Is it not more meaningful to capture time from initiation of referral to the conclusion of the (first) activity i.e. the generation of the report?

[7] We should change these words to reflect the terms used by the CPMS

				Are we capturing time in days?
	Average time between referral to ERN and diagnosis	Process	Again, we would need to use an agreed definition of Referral: propose propose 'creation of a case form via the CPMS and invitation of experts to review the case' The wording of this Indicator is problematic, as sometimes even with the best expertise, a diagnosis will not be forthcoming. And if it is, the quality – i.e. accuracy- of the diagnosis is important. Furthermore, not every patient will be seeking a diagnosis! Many will have a diagnosis, and the purpose of a consultation might be more about how to provide best care or treatment regime. One Gp proposed changing to: "average time from entry of patient into CPMS to end of diagnostic consultation in ERN (either clinical diagnosis or molecular diagnosis where possible and appropriate) Captures where no molecular diagnosis is possible and where patients may already have a clinical diagnosis	Might be better to omit this one. If it is retained, however, one should consider a change of wording e.g. **Average time between referral to ERN and accurate diagnosis, where relevant** OR more specifically, **Average time from initiation of a case review via the CPMS to end of diagnostic consultation in ERN (either clinical diagnosis or molecular diagnosis) where possible and appropriate**
	Number of patients seen/advised by ERN	Process	*Patients discussed within network* Again, need to better define 'seen/advised' here to distinguish the no. of patients referred for shared care in the ERN vs. the no. of patients seen by an HCP during its usual activities. As above, it is logical to define a patient seen/advised by the ERN as one for whom a case review is initiated via the CPMS.	Propose changing this to one of the following: **Number of patients referred for shared care within an ERN, via the CPMS**

			One Gp pointed out that numbers of patients entered to CPMS may be affected by resource available and complexity of patient cases	Or simply **Number of patient cases entered to the CPMS**
	Number of patients seen by the ERN HPCs		*As defined by the application and in order to maintain level of expertise* It is important to capture this data to demonstrate compliance with the disease-specific criteria established by each ERN on the application form – if a HCP claimed to see 300 patients with a particular group of diseases per year, it must collect data to demonstrate this.[8] One Gp proposed changing this to "total number of patients seen by all ERN HCPs – both those inside and outside of CPMS" Another Gp perceived an urgent need to define how we count these patients, particularly, how to distinguish between new patients and patients returning for a follow-up. Indeed, this breakdown is requested on the application form, and therefore HCPs will need to collect it anyway. Even the question of what one classes as patient is not so straightforward – for instance, if relatives are brought in, to assist in diagnosing the proband, are they counted too?[9]	If we accept the logic in capturing 'New' and 'Returning/Follow-up' patients here, one suggestion would be to replace this indicator with two, worded approximately as follows: **Total number of new patients seen by all ERN HCPs – both those inside and outside of CPMS** **Total number of follow-up/returning patients seen by all ERN HCPs – both those inside and outside of CPMS**

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[8] Although a little onerous, this is information HCPs agreed to collect. The figure provided for this Indicator would therefore be generated by the coordinator adding together the totals provided annually by each HCP. These total figures will be very important in demonstrating the sphere of influence of the ERNs, as arguably each patient visiting a specialised centre will benefit, tangibly or intangibly, from the growing expertise of the healthcare teams they encounter (growth stemming from the closer exchange of practices and experiences within the ERN framework).

[9] We will need to agree a definition here, as to how we 'count' a patient.

	Number of procedures performed within ERN	Process	*As defined by the application and in order to maintain level of expertise for each centre* The wording needs to be changed here, as an ERN will not perform a procedure. The ERN has no single site or premises from which to do this – procedures will always be performed at an HCP or local hospital or lab etc. It is important to better define what this indicator seeks to capture 1. Is it related to the specific criteria of the ERN applications, in which the Network defined the min. no. of procedures an HCP must perform to retain 'excellence' in a given field; or 2. Is it attempting to monitor the no. of procedures performed in HCPs ***directly resulting from*** virtual review of patients (i.e. occurring specifically as a result of ERN advice?) If option 2, it is important to remember that sometimes the specialised procedures will take place in centres outside of the ERN, as part of the mission to bring care closer to the patient. For this reason, interpretation 1 seems wisest.	If interpretation 1 (see left column) is more favourable, amend to the following: **Total number of procedures performed by ERN HCPs** Otherwise, specify if these should result from ERN 'advice'
	Level of patient satisfaction	Outcome	*Discuss on a common tool to measure patient satisfaction* Gps were unable to dedicate adequate time to this Indicator. However, one noted that EURORDIS should be closely involved in agreeing the tool to measure patient satisfaction. Another group pointed out that although this is an important Indicator, it will be very resource-intensive to collect... Crucially, we need to agree which patients we wish to survey regarding their satisfaction: 1. Option 1 would be just those referred to the ERN i.e. those whose cases are entered to the CPMS 2. Option 2 would be to assess the satisfaction of all patients receiving care at all HCPs in the ERN.	This Indicator needs particular attention, but first, an important distinction must be made: It is necessary to clarify which type of patient - patients of the ERN or patients of the HCP, **or both**- we wish to survey here (see options 1 or 2, left). Option 2 is surely logical, even considering the caveats in the comments

			The latter -i.e. a patient's experience of receiving care in any given clinic- should ideally be surveyed by HCPs already (this is one of the core criteria used to score HCP applications); however, this might be a good way to ensure all HCPs are capturing this data (and possibly harmonise how they do it). Most participants of the workshop *seemed* to assume we are most interested here in capturing patients' experience of shared care in an ERN.[10] If this is the case, this Indicator raises issues around the visibility of ERNs to patients, and how we increase the no. of patients ultimately receiving care via the ERNs. Finally, it is probably important to distinguish this Indicator -whichever option above we agree upon- as different to patients providing data on their conditions and their clinical outcomes.	section; however, this could also be a valuable opportunity to improve and harmonise the way in which patients with the same/similar conditions report satisfaction with the care received in heterogenous HCPs. Once the scope of this Indicator has been agreed, a dedicated piece of work will likely be required, to define a methodology for the data collection and agree a tool for collecting the information.
	~~**Number of complications/adverse events**~~	Outcome	*Important to agree on a common definition for complications* Both groups agreed that, if we retain this one, it is necessary to agree what we mean by a complication and it will be difficult to do this for all ERNs (which is the purpose of this exercise). However, many proposed that this be abandoned[11], as the ERN itself will not be performing procedures.	Proposed to abandon
	Average time from diagnosis to first treatment/surgery	Process	One Gp commented that this is an important indicator to measure progress of ERN towards "knowledge generation".	This Indicator needs further thought – what

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[10] VH – it strikes me that the amount of information patients can provide here will depend upon whether they are actively involved in the virtual review of their case or not… the general view seems to be that patients will *not* typically be directly involved in these reviews (e.g. they will not join real-time multi-expert consultations), and if this is the case, how much information can they really provide.

[11] Sample comment - 'very unclear what this means unless in specific situations of high expertise input- may need to be ERN specific'

			They recommend that this be a disease specific (presumably optional?) rather than common indicator, as it will not be applicable to all ERNs/conditions. (Due to the nature of the conditions involved, ERNs vary in terms of their orientation towards 'treatment/surgery', and in many diseases, there are no dedicated treatment options.) Some therefore proposed this one be considered an "indicator under development". The Gp suggested we should consider what we really want to learn from this indicator… (VH comment: For instance, it might be interesting/ more feasible to capture the time between a <u>recommendation</u> from the ERN for a patient to receive a particular procedure or surgery, and the actual performance of this procedure/surgery. If you tie this too closely to diagnosis followed by action, you will surely need to consider whether you count patients diagnosed before the ERN era, or only those diagnosed through that ERN. [12] Another Gp added the question of what resources are needed to support this – is the CPMS fit for purpose to achieve this (see previous footnote).	exactly do we wish to monitor here (see left)? Depending on the answer, we can rephrase (e.g. if we indeed wish to capture time from diagnosis to 1st treatment/surgery) we will need to add a '**where relevant**' option, and also define what we mean by 'treatment' (e.g. are we looking only at disease-modifying treatments?)
	Survival (i.e. after 1 year of diagnosis)	Outcome	*To be discussed carefully* Some participants commented that this is rather naïve – it is not something we are ready to begin capturing yet, and defining what we mean by survival will require many years. [13]	Many seemed in favour of 'shelving' this Indicator for now, for the reasons outlined in the column left

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[12] This would, however, entail follow-up of the patient's treatment being recorded in the CPMS. Collected longitudinally, this could help to demonstrate the -hopefully- growing influence of the advice of the ERN community, bearing in mind that procedures -if not part of the usual 'benefits basket'- may require extra approval from the relevant authorities, and it would be interesting to monitor how fully the advice of the ERN experts is actioned.

[13] VH Comment - What would one capture? The % of patients referred for shared care in the ERN alive after 1 year, after 5 years? Do we really want to use 'diagnosis' as the focus, or time from referral to ERN? What are we really seeking to capture?

			As above, one Gp highlighted this as an ‘Important indicator to measure progress of ERN towards “knowledge generation”’ - Recommend that this be classified as an “indicator under development” - Should consider what really want to learn from this indicator and what data and resources are needed to support this – is CPMS fit for purpose to achieve this?	
	Mortality	Outcome	*To be discussed carefully* Again, one Gp highlighted this as an ‘Important indicator to measure progress of ERN towards “knowledge generation”’ - Recommend that this be classified as an “indicator under development” Should consider what really want to learn from this indicator and what resources needed to support this – is CPMS fit for purpose to achieve this. And again, some felt this was rather naïve in present form, requiring further discussions	Many seemed in favour of ‘shelving’ this Indicator for now, for the reasons outlined in the column left
	Morbidity	Outcome	*To be discussed carefully* Again, one Gp judged this an important indicator to measure progress of ERN towards “knowledge generation” - Recommended that this be a disease specific and not common indicator – will not be applicable to all ERNs/conditions - Should consider what really want to learn from this indicator and what resources needed to support this – is CPMS fit for purpose to achieve this. Seems that this Indicator needs much more thought…	Many seemed in favour of ‘shelving’ this Indicator for now, for the reasons outlined in the column left
~~To optimise patient outcomes by combining skills of healthcare~~	**~~Number of Virtual consultations~~** **~~- with core MDT~~** **~~- with extended MDT~~**	Process	One Gp felt that this was already measured under “number of patients seen/advised by ERN” above, in section 2	Propose to discard as now covered in the better-defined section 2 Indicators
	~~Total number of patients discussed in~~	Process	As above, one Gp felt that this was already measured under “number of patients seen/advised by ERN” above, in section 2	Propose to discard as now covered in the better-

~~professionals involved and resources used~~	~~**Virtual consultations**~~			defined section 2 Indicators
To increase capacity of professionals to recognize and manage cases of rare and complex conditions and diseases within the scope of the ERN	**Number of educational training activities by ERN (on site and virtual) for:** **-Members of the network** **-Patients** **-Professionals outside the network**	Process	It is necessary to first define what once can include as 'training activities' here, as context for this indicator: can think broadly, including study visits, conferences, visiting professorships, Summer schools, clinical training, webinars, formal educational activities, virtual consultations. One group proposed that 'training activities' could encompass basically anything that the ERN either develops directly **or endorses**. VH – this flexibility is important, as surely we cannot include only events/activities fully funded by the ERN: in fields where some adequate training activities exist already, it does not make sense to duplicate. Therefore we need to add events which are relevant and take place anyway, but have 'ERN endorsement' (which presumably would come from a decision/vote by the Board). Do we want to make any provisos however, about training activities which the ERN is not creating anew (e.g. should the events bear the ERN logo, to be eligible for 'endorsement'?)	Wording of this indicator will need amendment to reflect the decision we take on how inclusive/broad to be here (i.e. whether to only include trainings with direct organisation/ funding by the ERN or all those with endorsement somehow (see comments) Depending on the decision, this could read something like: **Number of educational training activities organised by/endorsed by the ERN (on site and virtual) for:** **-Members of the network** **-Patients and families** **-Professionals outside the network**[14]
	Number of trained professionals	Outcome	Again, the previous issue needs to be resolved here first, in terms of what comes under the scope of a training activity of the ERN.	Once the above is resolved, this can be adapted to

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[14] VH - Might be helpful to list as exhaustively as possible the types of training activity one could envisage for each of these 3 categories, based on the comments in the left-hand column, and have this as context.

			Furthermore, one group proposed change from “trained professionals” to number of “people participating” (to include non-medical personnel and both junior and senior medical professionals). Some proposed counting by each type of professional.	read: **‘Total number of individuals participating to training activities organised by/endorsed by (TBD) the ERN’**
	Training evaluation	Outcome	*To discuss on common tool among ERNs for the evaluation of the trainings* The participants did not have time to address this	The participants did not have time to address this
To reinforce clinical research in the field rare and complex conditions and diseases by collecting data and carrying out research activities	**Number of project applications submitted as ERN**	Process	All groups raised the problem of the ERN not being a legal entity, and as such being unable to submit a project application per se. We also need to think of what sort of applications we mean here, as there are at least 2 broad options: 1. There will be grants which are **only** open to ERNs [15] 2. One could also theoretically count the no. of proposals submitted (for any type of grant) in which the expertise/resources of the **ERN** -as opposed to merely a single HCP- is emphasised somehow, to the extent it can justifiably be deemed to ‘involve the ERN’. If the latter is included, how would one judge that an application truly ‘involves’ the ERN? Do we mandate participation of a minimum % of HCPs from that ERN, or is simply mentioning the name of the ERN in the application sufficient? Unless the ERN becomes a legal entity, and can apply for any call under any programme, the former is the simplest and probably the	‘Submitted as ERN’ will not work. We need to decide whether, as per the comments column, we capture: 1. No. of applications submitted to ERN-only calls OR 2. No. of applications submitted on behalf of the ERN/with ERN involvement (or similar wording) If the second, we need to agree the qualifying criteria

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[15] Such as the CEF grant, and the registries call. Even so, the ERN itself cannot submit applications for these, at present; rather, it is the Coordinating HCP which submits, so the wording needs to be changed. If we merely count applications to such calls (i.e. those only open to ERNs) it is likely that the no. will be the same from ERN to ERN, as all Networks will apply to all possible calls.

			most realistic option at this stage…	for ‘with ERN involvement’ (see left)
	Number of grants received	Outcome	Same issues as the previous row. All groups raised the problem of the ERN not being a legal entity, and as such being unable to receive a grant. Presumably this would be directly related to the previous Indicator, and the criteria we establish for the former will be used here (i.e. we either limit this to grants for which only an ERN Coordinator is eligible to apply[16], or else we agree when an application is made ‘involving’ the ERN.	Wording to be made more precise, depending on the decision above
	Number of Clinical Trials with ERN participation	Outcome	Groups struggled to define ‘ERN participation’ here. VH - one could interpret this as a CT in which: • all ERN HCPs participate • one ERN HCP participates • Or anything in between! It is probably unlikely that any clinical trials will be conducted which *only* involve ERN HCPs (as to reach the critical mass of patients you typically need to aim as widely as possible) or involve *all* HCPs (as each ERN deals with a broad range of diseases and not all HCPs will be expert in the same diseases, within each Network). Thus we need to establish what ‘ERN participation’ means here. One group proposed changing this around to ‘number of ERN HCPs involved in clinical trials’ (VH – however, this will be all of them, surely, as it is a key requirement) One group proposed we define ‘a CT with ERN participation’ as a CT	The wording is fine here – the issues is how we define ‘ERN participation’. It is necessary to agree one of the options in the left column (or something else entirely). A good option seems: a CT involving at least 2 HCPs as trial sites, AND which is formally endorsed as within the scope/mission of the ERN by the Board/Steering Committee or equivalent

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[16] Such as the recent Registries call under the Health Programme and the CEF call

			involving at least 2 HCPs as trial sites, AND which is formally endorsed as within the scope/mission of the ERN by the Board/Steering Committee or equivalent Or -as a different Indicator perhaps?- one could collect the no. of enquiries directed at ERNs re. the **feasibility** of clinical trials	
	Number of ERN patients enrolled in Clinical Trials	Process	Here again you would have to define what is meant by an 'ERN patient'. One could use the definition proposed above, in section 2, but it might be more interesting -especially if we are seeking larger numbers – to count also (probably separately) the no. of patients visiting/cared for by each constituent HCP. The latter will give a much larger no., one group pointed out, but does it really have any bearing on the ERN?? Either way, having defined which patients we are focusing on, still one encounters problems. For instance, does one only count the CTs an ERN HCP actually enrols a patient to, or should we be routinely asking every patient how many CTs they are currently involved in?	We could keep this focused on the patents referred for shared care, and thus keep the wording as **'Number of ERN patients enrolled in Clinical Trials'**; however, we still need to agree on criteria outlined left.
	Number of patients of the ERN entered in shared registries (newly initiated registries or existing ones)	Process	This Indicator is fraught with difficulties. The data to be collected around registration is essential; however, there are many issues here, and it needs much more than a single row in the research category. For instance; • The tricky term again, as it stands, is 'no. of patients of the ERN' – are we only looking to count no. of patients referred for shared care (i.e. entered to the CPMS, as above section 2) who are enrolled into a registry? Or are we wishing to count all patients seen by any HCP who are enrolled into a registry? • Are we just interested in counting patients enrolled in registries henceforth, or also those already enrolled in registries (in which case we need to routinely ask patients?) • How does one define shared (do we only consider new, ERN-wide registries, or existing ones to which more than one HCP	We need more thought on this one – the topic and goal is VERY important, but we probably need to involve more actors to agree the best things to monitor regarding registries.

			participates?) - Do we seek to be more specific (as one group proposed) and ask about the levels of accessibility/interoperability of the registries? - Or, again as one Gp suggested, ERNs could count the number of diseases overall covered by a registry. - Biosamples should be counted here too perhaps? Remember that registries only collect data when there is a specific purpose…	
	Number of publications as ERN published in relevant scientific journals	Outcome	One group changed the wording to ‘peer reviewed’ journals here, for a start. Again, the challenge is agreeing what constitutes a publication of the ERN. VH - It is probably unrealistic to suggest that a publication can only be justifiably considered a publication ‘of the ERN’ if all HCPs have a named author! But at the other extreme, could a publication really be deemed to be ‘of the ERN’ if only one author from one HCP is mentioned, and there is no other involvement? One group recommended that some sort of endorsement from the Board/Steering Committee would be necessary here. So, perhaps one could set the criteria as either 1. ‘a fixed percentage of HCPs have a named author’ OR 2. ‘more than one HCP has a named author’	**Number of publications as ERN published in peer-reviewed journals** Then, it is necessary to agree criteria as per comments column

			AND couple this with the publication having received endorsement by one of the governance bodies above (i.e. to judge it in keeping with the scope and spirit of the ERN.)[17]	
	~~**Average and total impact factor of publications of the ERN**~~	Outcome	Not needed; impact factor may be misleading and not consistent across disease areas	Groups were in favour of abandoning this one
To ensure that all patients referred to ERNs have access to high quality healthcare services (title needs to be changed to differentiate it from section 2 -and logically- focus it more on guidelines)	**Number of new or of updated clinical guidelines (CG) adopted for the diseases and conditions within the scope of the ERN**	Structure	Firstly, we need to agree on how broadly we will define Clinical Guidelines here. CPGs are often quite field-specific. They can be of varying types, sometimes to inform the public and patient community, other times intended for specialists. The participants agreed on the importance of counting both new *and* updated guidelines. However, if we keep the current wording would we need to add some sort of clause here about level of ERN involvement in the generation/updating? Or does this not matter, and one could include in the tally also CPGs which were created without any ERN input? If the former, one could look to a definition such as 'generated/updated with involvement of at least 2 member HCPs' or else agree certain basic criteria for CPGs to be somehow 'endorsed' by the ERN. One group abandoned this Indicator, and replaced it with two more specific Indicators:	**One group felt the wording is reasonably okay for now, however depending on the decisions made (see left) may need refining.** **Another proposed breaking down into more specific Indicators:** • **'How many specialist clinical guidelines were produced in the ERN'**

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[17] VH - Another option might be to specify that 'ERN authorship' of a publication relies upon the use of ERN-exclusive resources, e.g. data from ERN-led registries or from the CPMS… but perhaps that would be premature, at present

			- ‘**How many specialist clinical guidelines were produced in the ERN’**[18] - **‘How many specialist care plans were produced in the ERN?’** VH comment – perhaps if we agree to break this one down, we should also collect the no. of layperson guidelines/guidance documents generated/updated by the ERN.	- **‘How many specialist care plans were produced in the ERN?’**
	% of CG developed according to internationally recognised methodology	Process	For instance patients and other experts should always be included – the RD-ACTION workshop on CPGs should result in criteria to support this. Do we already know which internationally recognised methodology is optimal here (e.g. isn’t ‘AGREE’ proscribed in the assessment criteria? If so, and there is really no option OTHER than generating in this way, this Indicator may be obsolete))	May be able to abandon this, if we know already at this stage what methodologies have been proscribed’ for the ERNs to use… If we keep it, the wording could perhaps be improved after the dedicated workshop on CPGs in December
	Adherence: % / Number of members within the network using the adopted CG	Process	How would this be proven? Self-reported, or would an audit be required (as one Group suspected)	If we keep, probably best to record % ? Would one include ‘Affiliated’ partners? Again, the RD-ACTION CPG Workshop in December will

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[18] This wording has the advantage of being more specific on the ERN involvement, as it says ‘produced in the ERN’ meaning the ERN will have to somehow ‘sign-off’ or endorse the final product

				support finalisation of this
	Compliance to clinical guidelines	Process	*Methodology to perform compliance studies should be agreed upon* The Gps had insufficient time to really delve into detail here. However, as above, to really assess compliance would involve site audits of some sort. One group suggested changing this to focus more on no. of specialist healthcare plans distributed to local teams (presumably advocating CPG practice?) We would need to distinguish carefully between adherence and compliance!	Insufficient discussion on this indicator – however, we would need to be clear as to how it differs from the previous Indicator.[19]
To guarantee that knowledge is spread outside the ERN so that more people can benefit from the ERN activities	**Participation to congresses and conferences representing the ERN and presenting ERN activities and results**	Process	People wished to add 'meetings' here. VH – it would probably be more logical to capture no. of such occasions when the ERN and/or its activities/results were presented[20]	Wording changed slightly to **'Participation to congresses / conferences/ meetings representing the ERN and presenting ERN activities and results'**
	Dissemination activities per: **- Type of organisation (patient organisation, scientific society)** **- Type of media**	Process	One group proposed adding more target groups here.	
	Number of individual ERN website hits	Process	Some participants felt that if one measures website hits, one will probably find the majority are internal ERN visitors. The group discussed in plenary how feasible it would be to capture both	Wording may change, depending on the results of EC investigation of what

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[19] For example - do we consider 'adherence' to record whether or not an HCP uses (or claims to use) a particular CPG (however minimally), thus resulting in a yes/no response? And 'compliance' to involve an assessment of the extent to which *all* the recommendations in that Guidance are followed?? Could become very complex

[20] Otherwise, you might end up counting people's participation each time, which might give you say 50 for a single conference!

			the no. of internal hits (i.e. from ERN HCPs and possibly also 'Affiliated' centres) and the no. of external visits. vs. external). Some commented that in their experience, one cannot easily determine which parts of a website a visitor has accessed and that a system which *does* provide such data takes a long time to set-up. However, others added that knowing the number of downloads of resources like Guidelines is very important and is data worth collecting and reporting here. It was agreed that we need to look at what is easily doable at present with existing systems (e.g. OrphaNews).	existing IT tools can capture and differentiate between at present; for instance, if feasible, it would be good to be able to capture no. of public document downloads (see below).
	Other type of documents produced by the network (e.g. Patient information leaflets) publications	Process	What type of documents are we referring to here? Presumably documents specifically about the ERN, i.e. excluding things like disease-specific information leaflets? Because if the latter *is* included, once again we would need to agree when a document with external (i.e. non-ERN involvement, from patient organisations or professional societies, say) becomes 'produced by the Network'	

Indicators can be raw numbers (e.g. number of cases per ERN), progress indicators requiring a numerator and a denominator (% completed) or means and medians, associated with a specific goal/target set by each ERN (see Annex).

It can also be envisaged that some initial indicators might be defined by the availability of data. If so, the indicators could be expected to change subsequently as more data becomes available and we move from the early implementation phase of the ERNs.

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 different intervals.

Who will input into the system

Both ERN coordinators and HCP will be responsible for providing data.

Who will monitor the system

ERN coordinators will use the system as an instrument to monitor their activities and identify areas for improvement. It will also be a great tool to prepare for the Evaluation process 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 but inevitably some of the indicators will be interlinked.

ANNEX

Understanding Indicators

For all identified indicators, we propose to run the exercise below with the ERN coordinators

INDICATOR TITLE	As specified in the table
Specific objective	As identified above
Type of indicator	Structure, Process, Outcome
What does it measure?	What the indicator measures (raw numbers, %, median)
Numerator – when applicable	Definition of numerator
Denominator – when applicable	Definition of denominator (target for each ERN)
Source of data	Surveys, databases, direct observation…
How often is it measured?	Frequency of recording data
Who is responsible for recording the data?	Coordinator? HCP?
Strengths and weaknesses	To be discussed by ERNs & MS

Example from the proposed indicators

INDICATOR TITLE	Number of patients see by the ERN HPCs
Specific objective	To improve diagnosis, treatment and follow-up of patients within the ERNs
Result	More patients getting appropriate treatment
Type of indicator	Process
What does it measure	Percentage reached
Numerator	Actual number of patients
Denominator	Estimated maximum number of new patients in a year (Application)
Source of data	Direct measurement
How often is it measured?	Data added throughout the year

Who is responsible for recording the data?	Each HCP should provide the number of patients visiting their hospital
Strengths and weaknesses	Weaknesses - Long intervals between measurements - First results available in a year Strengths - Unambiguiously interpretable -measurable indicator