Continuous Monitoring of ERNs

ERN Continuous Monitoring and Quality Improvement System (ERN CMQIS)

ERN indicators

November 2023

Version 8.1
(To be used in the data collection of 2024, ERN results of 2023)

ERN Continuous Monitoring Working Group

Version	Date	Comments
7.3	21.09.2020	Revision of indicators 3.1, 4.1, 4.2, 5.1, 5.2, 6.1, 7.1 Data collection of October 2020 (reporting period: Jan-Jun 2020)
7.3.1	14.10.2020	Summary of changes. Work in progress
7.4	24.02.2021	Editorial review Data collection of March 2021 (reporting period: Jan-Dec 2020)
7.4.1	01.03.2021	Work in progress Table of contents, minor rewordings,
7.4.2	08.07.2021	Work in progress Revision of indicator 5.1 by Monitoring Working Group
7.4.3	15.07.2021	Work in progress Further revision of indicator 5.1
7.5	07.09.2021	Editorial review Data collection of October 2021 (reporting period: Jan-Jun 2021)
7.5.1	15.09.2021	Corrected typo on indicator 1.3, discovered during the Monitoring Webinar of Oct 2021 Data collection of October 2021 (Jan-Jun 2021) and of March 2022 (Jan-Dec 2021)
7.5.2	9.06.2022	Work in Progress Data collection of 2023 (data of 2022 - reporting period: Jan-Dec 2022)
8.0	15.06.2023	New section (Introduction and Context) and KPIs for the ERN Direct Grants 2023-27. Overall revision of names and definitions.
8.0.1	22.06.2023	Work in progress. Adding more definitions for the Grant related KPIs Preparation for the data collection of 2024.
8.1	30.11.2023	Editorial review. Added glossary, legal references and minor revisions of definitions. Data collection of 2024 (reporting period: Jan-Dec 2023)
8.1.1	1.12.2023	Work-in-progress Revision of clarifications and examples by the Monitoring Working Group

Versioning convention (X.Y.Z):

- X: main version number - advances on every major revision of the document (new indicators, new sections, etc.)
- Y: sub-version number - advances on minor revisions of the document (update in the name or definition of an indicator, significant editorial change, etc.)
- Z: release number - for work-in-progress versions of the document

Contents

Introduction and context

The European Union's healthcare systems aim to provide high-quality, cost-effective care. This is particularly challenging in the case of rare or low-prevalence complex diseases that affect the daily lives of 30 million EU citizens. To address this inherent difficulty, the European Commission has launched the European Reference Networks policy initiative [1, art. 12], [2], [3]. This initiative is governed by a Board of Directors made up of Member States representatives and by the European Reference Network Coordinators Group.

European Reference Networks (ERNs) are virtual networks of healthcare providers across Europe. Currently there are 24 networks working in different areas of rare diseases. They aim to facilitate the cross-border sharing of knowledge between healthcare professionals on rare and complex diseases or conditions.

Core set of indicators

At the outset of the ERN initiative, the Board of Member States approved seven areas of intervention for ERNs. For each area, an objective and relevant indicators were defined and agreed by all ERNs, becoming what is known as the core set of ERN indicators. This set was used from 2017 to 2023, with only minor changes and clarifications to the definitions over time.

Data collection exercises

From 2017 to 2021, there were two data collection exercises per year - one in October, covering the period from January to June, and the other in March of the following year, covering the period from June to December. In 2020 and 2021, the March data collection has been adapted to cover the period from January to December of the previous year, but the October data collection has been retained.

From 2022, the October data collection has been discontinued. Currently, continuous monitoring of ERNs is based on a single data collection per year, in March, covering the period from January to December of the previous year.

Extended set of indicators

In September 2023, with the launch of multi-year direct grants for ERNs, several key performance indicators used to assess the fulfilment of grant obligations have been added to the common set of indicators. To simplify data collection and reporting, the data collection platform is being adapted accordingly and extended to all ERN members and affiliated partners.

This document provides the name and the approved definition for each indicator. It also gives examples and provides clarifications to questions received over time.

Legal references

1. Directive 2011/24/EU of the European Parliament and of the Council of 9 March 2011 on the application of patients' rights in cross-border healthcare. Available here (original text) and here (consolidated).
2. Commission Implementing Decision 2014/287/EU, of 10 March 2014, setting out criteria for establishing and evaluating European Reference Networks and their Members and for facilitating the exchange of information and expertise on establishing and evaluating such Networks. Available here.
3. Commission Implementing Decision (EU) 2019/1269, of 26 July 2019, amending the Implementing Decision 2014/287/EU. Available here.

Glossary

To avoid confusion and guarantee the full understanding of this document and concepts addressed, the following abbreviations and definitions apply:

HCP	Healthcare provider. While legally speaking HCP refers to any institution or professional that provides healthcare services, in practice this concept is usually applicable to institutions (hospitals in most of the cases).
HP	Healthcare professional. Any person accredited to provide healthcare services (clinicians, nurses, etc.).
ERN	European Reference Network. Virtual network of HCPs that cooperate to share knowledge and advance the state of the art in one or more specific thematic areas. ERNs are composed of members – full members and affiliated partners. All members are requested to participate in the yearly data collections.
CC	Clinical centre. A virtual entity that corresponds to the association of one ERN to one or more units/departments/services of one HCP.
CCC	Consortium clinical centre. A virtual entity that corresponds to the association of one ERN to one (or more) units/departments/services of more than one HCP.

ERNs intervention areas and specific objectives

The Board of Member States approved the following seven areas of intervention for ERNs:

General organisation and coordination

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

Patient Care

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

Multidisciplinary approach and sharing of knowledge within the ERN

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

Education and Training

Objective: 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

Contribution to research and innovation

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

Clinical guidelines

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

Communication and dissemination within the scope of the ERN activities

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

Core set of ERN Indicators

For each objective, one or more indicators were defined and agreed by all ERNs, becoming what is known as the *core set of ERN indicators*:

	Objective 1: To ensure that ERNs are operational and successfully carry out their activities	**Source**
1.1	Number of Member States represented in the ERN	EC
1.2	Number of full members of the ERN	EC
1.3	Number of affiliated partners of the ERN	EC
1.4	Number of patient organisations represented in the ERN	ERN
	Objective 2: To improve access to clinical advice, diagnosis, treatment, and follow-up of patients	
2.1	Number of new patients referred to the Health Care Providers of the ERN, with the diagnosis of a disease or condition that falls within the scope of the ERN	ERN
2.2	Number of patient cases discussed in the CPMS	EC
	Objective 3: To optimise patient outcomes by combining healthcare professionals' skills & resources used	
3.1	Number of CPMS outcome reports produced during the reporting period	EC
3.2	Days between enrolment in the CPMS and multidisciplinary clinical advice 3.2.a) non-urgent cases 3.2.b) urgent cases	EC
	Objective 4: To increase the 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 education/training activities not accruing higher education credits aimed at healthcare professionals delivered by the coordination teams or HCP members of the ERN	ERN
4.2	Number of formal educational activities that are accruing higher educational credits aimed at healthcare professionals delivered by the coordination teams or HCP members of the ERN	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	5.1.a) Number of Clinical Trials involving ERN members in at least two Member States 5.1.b) Number of Observational prospective studies / Observational cohort or case-control studies / Case-series studies involving ERN members in at least two Member States	ERN
5.2	Number of accepted peer-reviewed publications in accredited scientific journals falling within the scope of the ERN and acknowledging the ERN.	ERN
	Objective 6: To ensure that patients referred to ERNs have equal access to high and quality healthcare services	
6.1	Number of Clinical Practice Guidelines and other types of Clinical Decision-making Tools, adopted for diseases within the scope of the ERN	ERN
6.2.	6.2.a) Number of new Clinical Practice Guidelines written by the ERN and released during the reporting period 6.2.b) Number of other types of new Clinical Decision-making Tools (clinical consensus statements or consensus recommendations), written by the ERN and released during the reporting period	ERN

	Objective 7: To guarantee that knowledge, expertise and awareness 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	ERN
7.2	Number of individual ERN website hits	ERN
	Objective 8: Complex and long-term indicators which need further development	
8.1	Level of patient satisfaction (still t*o be developed*)	
8.2	Integration in the national health systems of Member States (still t*o be developed*)	

This set of indicators was in use until the data collection of 2023 (reporting period Jan-Dec 2022). As of the data collection of 2024 (reporting period Jan-Dec 2023) a new set of indicators is adopted, which includes the original core set with minor adaptations and most of the new KPIs used for grant reporting. This is known as the extended set of indicators and is described in the following section.

Extended set of ERN Indicators

This set of indicators, in use as of the data collection of 2024, is composed of the original core set of indicators with minor adaptations, plus most of the KPIs used to evaluate the accomplishment of the grant obligations. Not all KPIs are included in this set, only those that have a quantitative nature.

	Objective 1: To ensure that ERNs are operational and successfully carry out their activities	**Source**	**KPI**
1.1	Number of Member States represented in the ERN	EC	
1.2	Number of full members of the ERN	EC	
1.3	Number of affiliated partners of the ERN	EC	
1.4	Number of patient organisations represented in the ERN	ERN	
1.5	Global Performance of the members	ERN	1
	Objective 2: To improve access to clinical advice, diagnosis, treatment and follow-up of patients		
2.1	Number of new patients referred to the Health Care Providers of the ERN, with the diagnosis of a disease or condition that falls within the scope of the ERN	Members	6
2.2	Number of patients enrolled in the CPMS	EC	7
	Objective 3: To optimise patient outcomes by combining healthcare professionals' skills & resources used		
3.1	Number of CPMS outcome reports produced during the reporting period	EC	
3.2	Days between enrolment in the CPMS and multidisciplinary clinical advice 3.2.a) non-urgent cases 3.2.b) urgent cases	EC	
	Objective 4: To increase the capacity of professionals to recognize and manage cases of rare and complex conditions and diseases within the scope of the ERN		
4.1	4.1.a) Number of non-accredited education/training activities developed and delivered by the coordination team or by members of the ERN 4.1.b) Number of trainees who completed non-accredited training 4.1.c) Number of countries of origin of trainees who completed non-accredited training	ERN ERN ERN	20 21 22
4.2	4.2.a) Number of accredited education/training activities developed and delivered by the coordination team or by members of the ERN 4.2.b) Number of trainees who completed accredited training 4.2.c) Number of countries of origin of trainees who completed accredited training	ERN ERN ERN	17 18 19
	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	5.1.a) Number of Clinical Trials involving ERN members in at least two Member States 5.1.b) Number of Observational prospective studies, observational cohort or case-control studies and case-series studies involving ERN members in at least two Member States	ERN	
5.2	Number of accepted peer-reviewed publications in accredited scientific journals falling within the scope of the ERN and acknowledging the ERN.	ERN	
5.3	Number of new patients uploaded to the ERN registry	ERN	10
5.4	Percentage of the total ERN patients that are uploaded to the registry	ERN	11
5.5	Use of Orphacodes to code/classify patient cases	Members	16

	Objective 6: To ensure that patients referred to ERNs have equal access to high quality healthcare services		
6.1	Number of Clinical Practice Guidelines and other types of Clinical Decision-making Tools, adopted for diseases within the scope of the ERN	ERN	
6.2.	6.2.a) Number of new Clinical Practice Guidelines developed, updated, or appraised within the scope of the ERN.	ERN	23
	6.2.b) Number of other types of new Clinical Decision-making Tools (clinical consensus statements or consensus recommendations), developed, updated, or appraised within the scope of the ERN.	ERN	24
	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	ERN	
7.2	ERN web statistics 7.2.a) Number of new visitors 7.2.b) Number of returning visitors	ERN	2
7.3	7.3.a) Number of relevant documents downloaded from the ERN website	ERN	3
	7.3.b) Number of dissemination webinars		4
	7.3.c) Number of attendees to dissemination webinars		5
	Objective 8: Complex and long-term indicators which need further development		
8.1	Level of patient satisfaction (still to *be developed*)		
8.2	Integration in the national health systems of Member States (still to *be developed*)		

Notes:

- KPIs 8, 9, 12, 13, 14 and 15 are not included in the data collection platform because they don't have a quantitative expression but a qualitative one. They must be reported to HaDEA separately.
- KPI 2 is only partially included in indicator 7.2. A complete web analytics report must be submitted to HaDEA for the purpose of grant reporting.

For the data collection of 2024 only indicators 2.1 and 5.5 are directly uploaded in the data collection platform by the ERN members. The remaining KPIs still need to be reported to the ERNs outside of the platform. The platform is being expanded to include more indicators in future data collections. The objective is to include in the platform all grant KPIs that have a quantitative nature.

For the data collection of 2024 the reporting period corresponds to 2023 (January to December). Only indicators related to activities held during the reporting period should be reported. One example, among others, is indicator 1.5 (global performance score): if a network did not implement the scoring system in 2023, the indicator should be left blank and a comment inserted in the corresponding comment box.

IMPORTANT: as it was the case in the past, for audit purposes, the ERN indicators not coming directly from DG SANTE need to be validated by the publication of relevant evidence on the website of each ERN. Each ERN is responsible for securing the relevant evidence prior to the submission of the data or, at most, up to 30 calendar days after the submission.

ERN Grants KPIs

In this section, and just for information purposes, we provide the list of approved Key Performed indicators used in the new ERN grants.

To facilitate reporting, 19 of the 24 KPIs are included in the new data collection forms. For the purpose of grant reporting, the KPIs not included in the monitoring platform (those not having a quantitative expression) must be collected separately and submitted outside of the platform.

KPI	Designation	New?	In the new platform?
1	ERN Member Performance Indicator Score	Yes	Yes
2	ERN website statistics	No	Partially
3	Number of relevant documents downloaded from the ERN website	Yes	Yes
4	Number of dissemination webinars	Yes	Yes
5	Number of attendees to dissemination webinars	Yes	Yes
6	Number of new patients referred to the HCPs of the ERN with the diagnosis of a disease or condition that falls within the scope of the ERN	No	Yes
7	Number of patient cases discussed in the IT platform for cross-border clinical discussions (new CPMS)	No	Yes
8	Active participation in the new CPMS development, testing and roll-out	Yes	No
9	Implementation of the pilot scheme for reimbursement and drafting of recommendations for improvement	Yes	No
10	Number of new patients uploaded to the ERN registry	Yes	Yes
11	Percentage of the total ERN patients that are uploaded to the registry	Yes	Yes
12	Mapping of the registries/data sources in all the clinical units constituting each ERN (i.e., provide a list of the existing patient data sources in the clinical units of the ERN that are relevant for the ERN work on registries)	Yes	No
13	Metadata of the registries/data sources provided to the Directory of Registries and Central Metadata Repository of the Joint Research Centre	Yes	No
14	Design, proposition and implementation of a practical technical model to connect and make registries interoperable (using the European Rare Diseases Registry Infrastructure, tools and services)	Yes	No
15	Implementation of pseudonymisation, data linkage and data transfer services in line with JRC recommendation (SPIDER)	Yes	No
16	Mapping of the use of orphacodes in all clinical units	Yes	Yes
17	Number of accredited Training Courses developed by the ERN	No	Yes
18	Number of trainees who completed accredited Training	Yes	Yes
19	Number of countries of origin of trainees who completed accredited	Yes	Yes
20	Number of non-accredited training courses developed by the ERN	No	Yes
21	Number of trainees who completed the training	Yes	Yes
22	Number of countries of origin of trainees who completed the Training	Yes	Yes
23	Number of Clinical Practice Guidelines developed, updated, or appraised	No	Yes
24	Number of Clinical Decision Support Tools developed, updated, or appraised	No	Yes

Of the 24 grant KPIs only 6 are not included in the new platform and must be reported separately. KPI 2 (web analytics) is partially included and requires a separate web analytics report for the purpose of grant reporting.

Definitions of ERN Indicators

In this section we provide the approved definitions of each indicator. In the data collection forms these definitions appear when hovering over the indicator name. Some indicators correspond to the grant KPIs.

Objective 1: ensure that ERNs are operational and successfully carry out their organisational activities			
Ind	**Name**	**Definition**	**Source**
1.1	Number of Member States represented in the ERN	The number of Member States within the EEA covered by Directive 24/201 with at least one Health Care Provider participating as full member or affiliated partner in the ERN	EC
1.2	Number of full members of the ERN	The number of Health Care Providers participating as full member in the ERN	EC
1.3	Number of affiliated partners of the ERN	The number of Health Care Providers participating as affiliated partner in the ERN	EC
1.4	Number of patient organisations represented in the ERN	The number of patient associations represented by one or more persons actively involved in the ERN. Patients may work within an ERN in many ways to capture their voices and their needs	ERN
1.5 (KPI 1)	Global Performance Score	Median, in percentage, of the combined performance scores of all the members of the ERN. Corresponds to KPI 1 of the call for grants 2023-2027.	ERN

Objective 2: improve access to clinical advice, diagnosis, treatment, and follow-up of patients			
Ind	**Name**	**Definition**	**Source**
2.1 (KPI 6)	Number of new patients referred to the Health Care Providers of the ERN, with the diagnosis of a disease or condition that falls within the scope of the ERN	The number of patients attending the ERNs' Health Care Providers for the first time during the reporting period, whose disease or condition falls within the scope of the ERN, whatever their age. Includes visits to outpatient's clinics, hospital discharges and emergencies, coming from national and international referrals. Corresponds to KPI 6 of the call for grants 2023-2027.	ERN Members
2.2 (KPI 7)	Number of patients enrolled in the CPMS	The number of new patients enrolled in the CPMS during the reporting period, regardless of the status of the case discussions. Corresponds to KPI 7 of the call for grants 2023-2027.	EC (CPMS)

Objective 3: optimise patient outcomes by combining healthcare professionals' skills & resources used			
Ind	**Name**	**Definition**	**Source**
3.1	Number of CPMS outcome reports produced during the reporting period	The number of case discussions, of new or recurring patients, that resulted in the production of an outcome report	EC (CPMS)
3.2	Days to multidisciplinary clinical advice: a) for non-urgent cases b) for urgent cases	Days between start of discussions in the CPMS and multidisciplinary clinical advice either for non-urgent cases or for urgent cases. Only applicable to discussions that resulted in the production of an outcome report	EC (CPMS)

Objective 4: increase the capacity of professionals to recognize and manage cases of rare and complex conditions and diseases within the scope of the ERN			
Ind	**Name**	**Definition**	**Source**
4.1 (KPI 20)	a) Number of non-accredited education/training activities developed and delivered by the coordination team or by members of the ERN	The total number of unique non-accredited education/training activities aimed at healthcare professionals, students, or patients, created by the ERN coordination team, Full Members, Affiliated Partners, ePAGs/Patient Organisations of the ERN and delivered during the reporting period. Can be online or physical. Corresponds to KPI 20 of the call for grants 2023-2027.	ERN
(KPI 21)	b) Number of trainees who completed non-accredited training	Number of trainees who completed non-accredited training. Corresponds to KPI 21 of the call for grants 2023-2027.	ERN
(KPI 22)	c) Number of countries of origin of trainees who completed non-accredited training	Number of countries of origin of trainees who completed non-accredited training. Corresponds to KPI 22 of the call for grants 2023-2027.	ERN
4.2 (KPI 17)	a) Number of accredited education/training activities developed and delivered by the coordination team or by members of the ERN	The total number of unique accredited education/training activities aimed at healthcare professionals, students, or patients, created by the ERN coordination team, Full Members, Affiliated Partners, ePAGs/Patient Organisations of the ERN and delivered during the reporting period. Can be online or physical. CME and ECTS credits are both eligible. Corresponds to KPI 17 of the call for grants 2023-2027.	ERN
(KPI 18)	b) Number of trainees who completed accredited training	Number of trainees who completed accredited training. Corresponds to KPI 18 of the call for grants 2023-2027.	ERN
(KPI 19)	c) Number of countries of origin of trainees who completed accredited training	Number of countries of origin of trainees who completed accredited training. Corresponds to KPI 19 of the call for grants 2023-2027.	ERN

Objective 5: increase the capacity of professionals to recognize and manage cases of rare and complex conditions and diseases within the scope of the ERN			
Ind	**Name**	**Definition**	**Source**
5.1	a) Number of Clinical Trials involving ERN members in at least two Member States	The number of unique clinical trials that involve ERN members from two different Member States and acknowledge the ERN, either finalized or ongoing during the reporting period.	ERN
	b) Number of Observational prospective studies, observational cohort or case-control studies and case-series studies involving ERN members in at least two Member States	The number of unique observational prospective studies (including academic and Industry driven studies), observational cohort studies, case control studies or case-series studies that involve ERN members from two different Member States and acknowledge the ERN, either finalized or ongoing during the reporting period.	ERN
5.2	Number of accepted peer-reviewed publications in accredited scientific journals falling within the scope of the ERN and acknowledging the ERN	The number of unique peer-reviewed publications accepted for publication in scientific journals during the reporting period regarding disease-groups falling within the scope of the ERN. Publications should be PubMed accredited scientific journals and involve as major contributors Health Care Providers from at least two different Member States within the ERN, and which include an explicit acknowledgement of the ERN such as *"This work is generated within the European Reference Network for…" or "This work is supported by the European Reference Network for…"*	ERN
5.3 (KPI 10)	Number of new patients uploaded to the ERN registry	The number of new unique patients uploaded to the ERN managed registry or registries. Corresponds to KPI 10 of the call for grants 2023-2027.	ERN
5.4 (KPI 11)	Percentage of the total ERN patients that are uploaded to the registry	Percentage of the total ERN patients that are uploaded to the ERN managed registry or registries. Corresponds to KPI 11 of the call for grants 2023-2027.	ERN
5.5 (KPI 16)	Use of Orphacodes to code/classify patient cases	Acknowledgement that a clinical centre uses orphacodes to code/classify patient cases in case the use of orphacodes is a relevant goal for the ERN. Corresponds to KPI 16 of the call for grants 2023-2027.	ERN Members

Objective 6: ensure that patients referred to ERNs have equal access to high quality healthcare services			
Ind	**Name**	**Definition**	**Source**
6.1	Number of Clinical Practice Guidelines and other types of Clinical Decision-making Tools, adopted for diseases within the scope of the ERN	The number of Clinical Practice Guidelines and other types of Clinical Decision Making Tools, such as clinical consensus recommendations for disease areas within the scope of the ERN not necessarily developed by the ERN but that were formally endorsed and adopted by the ERN Board and are publicly available (e.g. on the ERN website).	ERN
6.2 (KPI 23)	a) Number of new Clinical Practice Guidelines developed, updated, or appraised within the scope of the ERN.	The number of Clinical Practice Guidelines (CPG) developed updated, or appraised by the ERN, involving Health Care Providers from at least two different Member States within the ERN. They should acknowledge the ERN, for diseases within the scope of the ERN where no guidelines existed previously and be according to an evidence based recognized methodology. The new CPGs can be in progress or finalised during the reporting period. Corresponds to KPI 23 of the call for grants 2023-2027.	ERN
(KPI 24)	b) Number of other types of new Clinical Decision-making Tools developed, updated, or appraised within the scope of the ERN.	The number Clinical Decision Support Tools (CDST: clinical consensus statements or consensus recommendations), developed, updated, or appraised by the ERN involving Health Care Providers from at least two different Member States within the ERN. They should acknowledge the ERN, for diseases within the scope of the ERN where no tools existed previously and be according to an evidence based recognized methodology. The new CDST can be in progress or finalised during the reporting period. Corresponds to KPI 24 of the call for grants 2023-2027.	ERN

Objective 7: guarantee that knowledge, expertise, and awareness are spread outside the ERN so that more people can benefit from the ERN activities			
Ind	**Name**	**Definition**	**Source**
7.1	Number of congresses, conferences, and meetings at which the ERN activities and results were presented	The total number of congresses, conferences, and meetings at which the ERN activities and results were presented via a dedicated slot in the program/agenda, acknowledging the ERN, during the reporting period.	ERN
7.2 (KPI 2)	a) Number of new visitors to the ERN website	Number of new visitors to the ERN website pages, according to the web analytics report. Is part of KPI 2 of the call for grants 2023-2027.	ERN
	b) Number of returning visitors to the ERN website	Number of returning visitors to the ERN website pages, according to the web analytics report. Is part of KPI 2 of the call for grants 2023-2027.	ERN
7.3 (KPI 3)	a) Number of relevant documents downloaded from the ERN website	Number of downloads of the most relevant and important documents (Guidelines, Training Materials, etc. Not leaflets, flyers, etc.). Corresponds to KPI 3 of the call for grants 2023-2027.	ERN
(KPI 4)	b) Number of dissemination webinars	Number of unique dissemination webinars (non-technical) addressing the public at large, delivered during the reporting period. Corresponds to KPI 4 of the call for grants 2023-2027.	ERN
(KPI 5)	c) Number of attendees to dissemination webinars	Total number of attendees to all editions of dissemination webinars during the reporting period. Corresponds to KPI 5 of the call for grants 2023-2027.	ERN

Clarifications and examples

Indicator 1.4 - Number of patient organisations represented in the ERNs

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 associations 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, sectorial or thematic patient associations meetings, etc.) should also be counted.

- 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 multidisease 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.

Indicator 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

- New patients are those that have attended or been referred to the healthcare provider, within the specified timeframe and having a certified diagnosis of rare disease. These patients should not have been previously included in the patient information system of the healthcare provider.
- Patients who have not obtained yet a diagnosis should not be taken into account.
- 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 between the ERNs on the type of contact with the healthcare provider. 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.
- It would be important to consider the aggregated number of patients at the 31 of December of the previous year of the reported period.

Indicator 4.1 - Number of education/training activities not accruing higher education credits aimed at healthcare professionals delivered by the coordination teams or HCP members of the ERN

- Education/training activities not accruing higher educational credits that are delivered by an ERN within the specified timeframe and are publically available (eg on websites or ERN educational platforms) should be counted.
- If an educational activity with the same content is delivered 3 times in one year, this should be counted as 1.
- Educational activities should feature the ERN logo.

Indicator 4.2 - Number of formal educational activities (i.e. those accruing higher educational credits) aimed at healthcare professionals organised by the ERN

- 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, member or non-members of the Networks, organised (including co-organisation or with important contribution) by the coordinating healthcare provider of the ERN or by one or more healthcare providers of the ERN.
- The activity should acknowledge the ERN participation (including the logo of the ERN) within the specified time period.
- Accredited digital educational activities should be included, including accredited Webinars and eLearning courses.
- **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)

Indicator 5.1 - Number of clinical trials, observational prospective studies, observational cohort or case-control studies and case-series studies within the ERN (involving ERN members in at least two Member States).

- this indicator is asking for the number of trials, observational prospective studies, observational cohort studies, case-control studies or case series studies that

  a) Involve healthcare providers within an ERN and b) includes an acknowledgement of the ERN.

- These qualifying criteria can be presented together or in different documents.
- Please provide a reference for each study on the ERN website: https://clinicaltrials.gov/ct2/home and EMA or EU trials registry systems 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 healthcare providers 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:

https://clinicaltrials.gov/ct2/show/study/NCT03189875?id=NCT02971683+OR+NCT03189875+OR+NCT02419365+OR+NCT03857880&rank=2&show_locs=Y&load=cart#locn

After several discussions, the Monitoring Working Group decided at the meeting of 1 July 2021 to adapt Indicator 5.1 on research to include a broader range of ERN research activities, more adequate to the needs of some networks.

In some cases observational cohort studies are conducted, although not purely prospective in design but with a retrospective or mixed cohort design (with both retrospective and prospective phases). They are used with appropriate methodologies to anticipate on the limitations of the study designs.

Case-control studies and case series are used for the identification of new genetic predispositions or (genetic) risk factors, which is an important aspect of research in the field of (unexplained) hereditary or genetic diseases.

Considering the evidence-based medicine pyramid, these study designs score lower however they do contribute to the relevant evidence for certain aspects of the work of some ERNs.

Indicator 5.2 - Number of accepted peer-reviewed publications in scientific journals regarding disease-groups within the ERN and which acknowledge the ERN.

For counting purposes, ERNs should only be counting those publications that include an explicit acknowledgment or reference the ERN's involvement such as **"This work is generated within the European Reference Network for …" or "This work is supported by the ERN for ….".** If the support is not financial, "(not financially)" can be added in brackets after the word "supporting" for additional clarification. This could be the case, for example, for ERNs that are involved in the Solve-RD or other similar European projects where ERN clinicians have been involved in publications but no ERN funding has been used.

The acknowledgment for collaboration can be included in the acknowledgement section and could follow this example:

"This research is supported (not financially) by the European Reference Network on Genetic Tumour Risk Syndromes (ERN GENTURIS)—Project ID No 739547. ERN GENTURIS is partly co-funded by the European Union within the framework of the Third Health Programme "ERN-2016—Framework Partnership Agreement 2017–2021"

The figure captured here should be clearly linked to the ERN and its activities.

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.

Indicator 6.1 - Number of Clinical Practice Guidelines and other types of Clinical Decision Making Tools, adopted for diseases within the scope of the ERN.

- "The ERN has adopted the CPG or Clinical Decision Making Tools" means that the tools are publically available and all the healthcare providers 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 Clinical Practice Guidelines (CPG) and other Clinical Decision Support Tools (CDST) based on consensus techniques to be counted shall be those adopted on the measured timeframe by the ERN (eg agreed by the ERN Board), not when they are published.
- The adoption of CPGs within an ERN could be defined, for example by means of an official endorsement of the Board of the ERN.

Indicator 6.2 – Number of a. new Clinical Practice Guidelines and b. other types of new Clinical Decision Making Tools (clinical consensus statements or consensus recommendations), written by the ERN in the specified time period.

- 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.
- 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 on the measured timeframe by the ERN (eg agreed by the ERN Board).
- 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 considered crucial
- In many cases the role of ERNs would be to collect the evidence that will represent the baseline 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 good 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.

Indicator 7.1 - Number of congresses/conferences/meetings at which the ERN activities and results were presented

- The aim of this indicator is to capture the dissemination activities of the ERNs.
- The ERN and its activities should be the focus of the presentations.
- If multiple qualifying presentations are given at an event, this should only be counted once.

Indictor 7.2 - Number of individual ERN website hits

- There are different web analytics tools available on the market that are suitable to implement this indicator.
- Please pay attention to GDPR compliance. For example, the popular suite “google analytics” (GA) is not compliant to the GDPR.
- Many alternatives to GA are available - please have a look at https://www.semrush.com/blog/google-analytics-alternatives. We recommend using open-source tools.