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Study on Enhancing implementation of the Cross-Border Healthcare Directive 2011/24/EU to ensure patient rights in the EU

Intervention logic and associated indicators for evaluation purposes

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1 Introduction

The EU Directive (2011/24/EU) on the application of patients' rights in cross-border healthcare came into force in April 2011 and was due to be transposed by Member States into national law by October 2013. The Directive clarifies the rights of patients to seek reimbursement for healthcare received in another Member State. The EU Directive has also considered European cooperation in healthcare and importantly the establishment of the European Reference Networks for rare and low prevalence complex diseases.

In preparation for the forthcoming evaluation of the Directive after almost 10 years since adoption, the European Commission has commissioned **a study to enhance the implementation of Directive**. Since evaluation framework of EU policies, programmes and legislation rely on an "**intervention logic**" and such a logic model was not developed at the time of impact assessment accompanying the proposal for the Directive in 2008, this had to be developed as part of the current preparatory study. In addition, the development of **qualitative and quantitative indicators** linked to the intervention logic of the Directive was requested so that to facilitate the evaluation of the Directive.

It is important to emphasise that developing an intervention logic retrospectively must draw a clear line and distinguish between **how the Directive was *expected* to work at the outset** and how in practice it *did work*. This baseline framework will provide the future evaluator of the Directive a tool to identify not only intended but also unintended (positive and possibly negative) effects of the Directive.

The approach adopted follows iterative steps: First, available document sources were analysed including the Commission's impact assessment, the text of the Directive itself and the early evaluative study of the Directive[1],[2]. The preliminary intervention logic model and indicator analysis was then presented to the Commission and revised based on feedback. It was then presented to key stakeholders for critical review and discussed in the form of targeted interviews. Finally, the intervention logic and indicators were presented to a wider group of stakeholders during an **online stakeholder workshop on 20 May 2021 to invite feedback on the work and discuss the indicator set for evaluation purposes**.

The following sections of the draft analytical report provide a brief introduction to the literature search and the process for the stakeholder workshop. This is followed by an overview of the concept of "intervention logic" to facilitate the interpretation of the analytical work presented. It should be remarked that the analytical work involved distinct approaches to cover patient rights (articles 1-11); and separately cooperation in rare diseases and the setting up of the European Reference Networks (articles 12-13). Consequently, the intervention logic and associated shortlist of indicators are presented in two parts:

- The first part focuses on the patient rights of the Directive;
- The second part includes cooperation in rare diseases and the functioning of the European Reference Networks (ERNs).

[1] Proposal for a Directive of the European Parliament and of the Council on the application of patients' rights in cross-border healthcare. Impact assessment (2008).
[2] Evaluative study on the cross-border healthcare Directive (2011/24/EU).

1.1 Literature review and targeted interviews

The development of the Intervention Logic relied on available reports from the Commission website on Cross-Border Healthcare Directive and academic/grey literature on the problems and needs at the time of the development of the Directive. We used a rapid evidence assessment using keywords in bibliometric databases (Scopus and Google Scholar) as well as search engines to identify such additional reports. However, these reports only contributed to parts of the intervention logic but left gaps that had to be explored using targeted interviews.

Literature review was also useful to understand available healthcare indicators. For example, the OECD has an annual "Health at a glance" report[3] which presents comparable data and trends over time on population health and health system performance, including in the EU. For rare diseases, results of the rare diseases task force working group on health indicators[4], the EUCERD recommendations on ERNs[5] and the specific literature related to the cross-border directive were helpful.

0 provides a list of references that was used for developing the intervention logic and indictor review, while 0 provides an overview of the stakeholders interviewed in order to provide insight to complete data gaps.

1.2 Stakeholder workshop

A stakeholder workshop was conducted which had the following main objectives:

- Present the Directive's intervention logic and longlist of indicators;
- Have an interactive poll and brief discussion on the relevance and feasibility of the longlist of potential indicators for evaluation purposes;
- Provide the European Commission with indications as to the relevance and feasibility of shortlisted key indicators.

The technical workshop focussed on the **evaluation framework** and it did not represent a forum for discussion on how the Directive functions currently. This will be the subject of a separate evaluative study.

This study aimed to build on this prior body of evidence and identify a mix of quantitative and qualitative indicators that may be used in the first instance for the forthcoming evaluation of the Directive. **Quantitative** indicators (i.e. numbers) and **qualitative** indicators (i.e. views and perceptions) together explore objective trends and explain the mechanism of change. These indicators were then linked to the Directive's intervention logic and structured along the standard **evaluations criteria of Effectiveness, Efficiency, Relevance, Coherence and EU added value.**

The associated indicators were assessed against the RACER criteria (relevant, accepted, credible, easy and robust), in line with the Commission's Better Regulation guidelines and toolbox. During the workshop with stakeholders, there was a focus on *relevance* and *feasibility* and discussion whether the indicators are

- *Relevant* for the Directive's objectives?
- *Feasible* to collect data to populate the indicators?

[3] Health at a Glance 2020: OECD Indicators, OECD.
[4] https://webgate.ec.europa.eu/chafea_pdb/assets/files/pdb/20082291/20082291_d04_01_oth_en_ps.pdf.
[5] https://ec.europa.eu/health/sites/default/files/ern/docs/eucerd_rd_ern_en_0.pdf.

On feasibility, it is crucial that the shortlisted indicator set proposed here is of practical use for evaluation (and possibly future monitoring) purposes. Therefore, the workshop explored whether (comparable) data collection is an easy process across all Member States. An online poll was used during the stakeholder workshop to help rank the draft set of indicators and shortlist the key indicators. The workshop also attempted to explore if it is possible to define a baseline (e.g. using data from 2010 if available) and provide an expected target.

The proposed shortlist of indicators for the forthcoming evaluation of the Directive can be found in sections 2.10 (for patient rights) and section 3.11 (for ERNs).
Note however that the shortlisted indicators were submitted to the European Commission for further reflections and the final list of indicators to be used for the future evaluation of the Directive may be amended based on additional considerations.

1.3 The concept of an intervention logic

An intervention logic is a key aspect of an evaluation design and summarises **how an intervention** (in this case the Directive) **was *expected* to work**. It shows the different steps involved in the implementation of the Directive and highlights expected cause and effect relationships. It thus seeks to answer the following questions:

- What were the **needs** that triggered the Directive? What problems were the intervention meant to solve?
- What are the **objectives** that link the needs analysis to the Directive? What should be achieved?
- What **inputs** were expected to be used? (Inputs can cover resources such as staff, time, financial support, or equipment)
- What **activities** were expected to take place? What obligations were set or what provisions were expected to be put in place by the Directive?
- What are the immediate and intended **outputs** of the activities foreseen by the Directive?
- What are the medium-term **outcomes** expected of the implementation of the Directive?
- What are the long-term **impacts** expected of the implementation of the Directive?
- What are the **external factors** that potentially influence / confound the performance of the implementation of the Directive?
- What are the underlying **assumptions** and dependencies in the causal logic chain so that the impacts can emerge due to the implementation of the Directive?

The intervention logic can be depicted as a diagram and expanded in a descriptive text as to provide a 'baseline' for future evaluations. This then serves to support the assessment of evaluation criteria of effectiveness, efficiency, relevance, coherence and EU added value for the Directive.

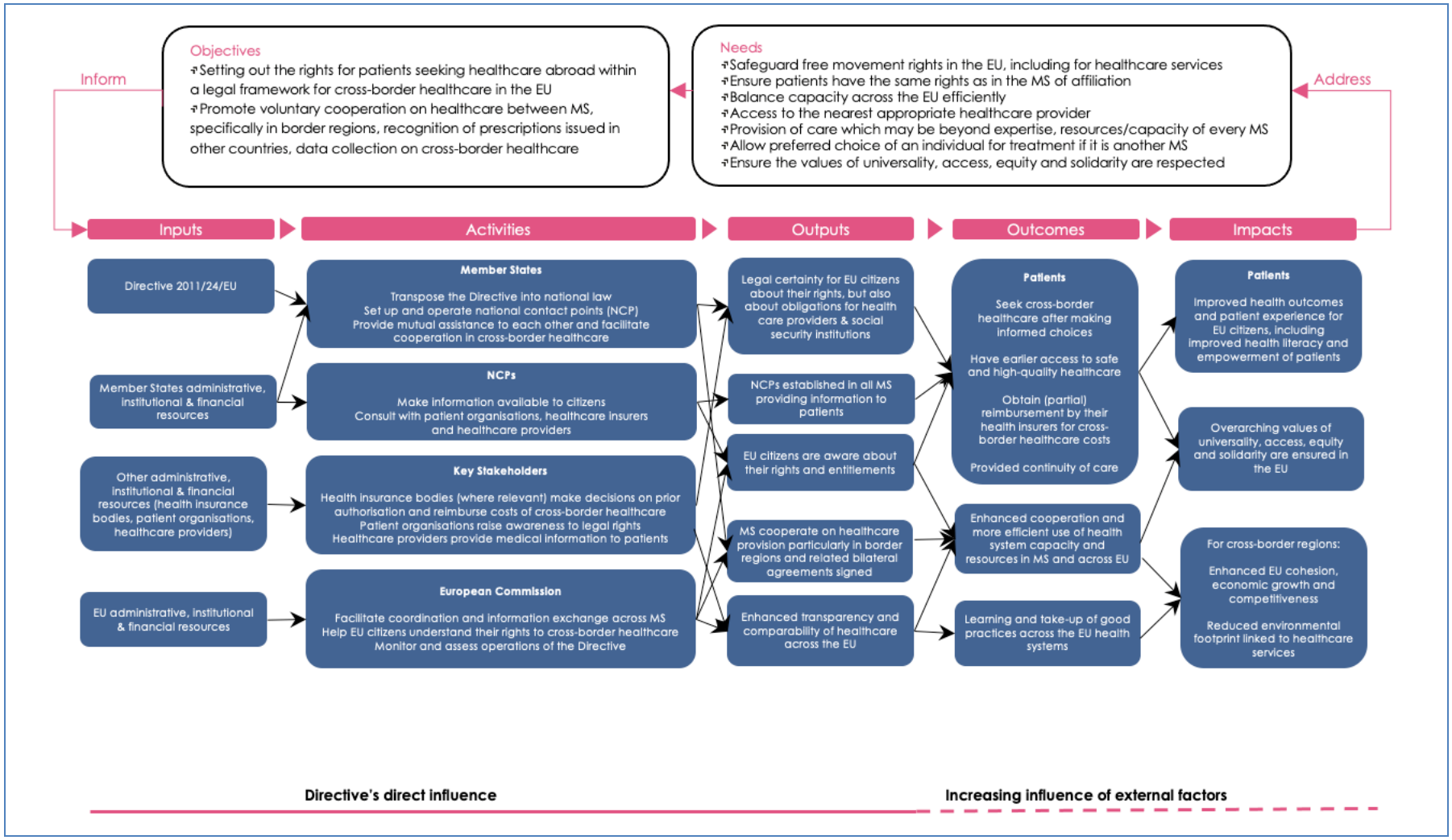
2 Patient rights

2.1 Introduction

The Directive 2011/24/EU on patient rights in cross-border healthcare was drawn up to codify judgments of the European Court of Justice which enable all EU citizens' access to safe and high-quality healthcare in another Member State. In addition, it also promotes cross-border cooperation in healthcare between Member States and with the support of the European Commission for the benefit of EU citizens.

The following diagram provides an overview of the needs identified prior to 2011 and the high-level objectives of the Directive that respond to these needs. The Directive was implemented with inputs and through a series of activities which were expected to lead to various direct outputs, and with time related outcomes and impacts. The influence of the Directive on the outcomes and impacts are expected to be 'contributory' among the number of external factors that are identified in subsequent sections.

Figure 2.1 Intervention logic of the Directive 2011/24/EU Articles 1-11 on patients' rights



Source: Technopolis Group.

2.2 Needs

The Regulation 883/2004 on the coordination of social security systems had foreseen under specific circumstances rights for EU citizens for treatment in another Member State. Under the Regulation, which is based on free movement of workers, patients are reimbursed for healthcare as if they were insured under the social security system of the Member State where treatment was received for necessary unplanned and pre-authorised planned healthcare. However, following a number of Court rulings on individual cases, new legislation was needed to safeguard free movement of healthcare services in the EU and to meet patient needs.

Most people receive healthcare in their own country and the vast majority will continue to do so. However, in certain circumstances, patients may prefer to seek some forms of healthcare in another Member State, for example for highly specialised care, or in border regions where the nearest appropriate healthcare provider may be across the border. Some patients may wish to be treated abroad to be close to their family members who are residing in another Member State, or in order to have access to a different method of treatment than the one provided in the Member State of affiliation (where the patient is insured) or because they believe that they will receive better quality healthcare in another Member State.[6]

Furthermore, in a system where there is free movement of patients[7] (based on free movement of workers, services and goods), capacity of healthcare[8] could be balanced more efficiently across the EU (e.g. for certain treatments).

2.3 Objectives

2.3.1 Overall objectives

Therefore, the Directive has two high-level objectives:

- To set out the rights for patients seeking healthcare abroad within a clear legal framework for cross-border healthcare in the EU;
- To promote voluntary cooperation on healthcare between Member States, specifically between neighbouring countries in border regions, recognition of prescriptions issued in other countries, data collection on cross-border healthcare.

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6 See Directive (39).

7 Directive (29) It is appropriate to require that also patients who seek healthcare in another Member State in other circumstances than those provided for in Regulation (EC) No 883/2004 should be able to benefit from the principles of free movement of patients, services and goods in accordance with the TFEU and with this Directive.

8 The 2008 Impact Assessment's problem definition context stated: "However, there are situations when cross-border healthcare can be more appropriate, such as: […] or in cases of lack of capacity, where local services are unable to provide the appropriate healthcare and there is capacity available in another Member State".
Seasonal effects are noted in the Impact Assessment (2008) quoting the consultation results from 2006: "noticeable are the travels of northern seniors towards southern Europe, for holidays, or for seasonal stay, as a second home, or even as a permanent residence".
In the 2012 Impact Assessment (recognition of medical prescriptions): "It is likely that geographic, seasonal and demographic patterns are at play."

2.3.2 Specific objectives

Linked to these high-level objectives, the Directive targets specific objectives:

- Establish common principles and clarify responsibilities of Member States and healthcare providers for cross-border healthcare (e.g. healthcare providers need to make information accessible to patients e.g. on available treatment and costs);
- Clarify entitlements of patients to have healthcare in another Member State;
- Ensure rights to reimbursement (under certain conditions) for healthcare abroad can be used in practice (e.g. through transparent and timely processes);
- Ensure high-quality, safe and efficient cross-border healthcare;
- Ensure continuity of care between Member State of treatment and Member State of affiliation (e.g. through the entitlement to a copy of the medical record of treatment, medical follow-ups, and mutual recognition of prescriptions).

2.3.3 Operational objectives

On the more operational level, the Directive has set out:

- To ensure common, clear and enforceable rules regarding the necessary criteria, processes and mechanisms covering cross-border healthcare and reimbursement practices;
- To ensure information is available and accessible to patients about cross-border healthcare and how it relates to the social security regulations.

2.4 Inputs

There was no documentary evidence on the level of specific resources foreseen for the implementation of the Directive. Nevertheless, based on the main stakeholder groups and their responsibilities the following can be reasonably expected:

- Administrative, institutional & financial resources provided by the **European Union**. More specifically the officers of the European Commission would support coordination, consultation, information exchange, and execute enforcement of the Directive;
- Administrative, institutional & financial resources by the **Member States** at national and regional levels, e.g. transposition of the Directive, establishment (where there had not been national or regional already) and operating National Contact Points (NCPs), monitoring data collection and reporting to the Commission;
- Administrative & financial resources by third parties, especially **health insurance bodies** responsible for the reimbursement of costs incurred for healthcare in another EU country and, in a number of EU countries, for decisions on the prior authorisation of healthcare in another EU country. In addition, **patient organisations** (for awareness raising) and **healthcare providers** (for information provision requirements and additional administrative processes) contribute relevant resources.

2.5 Activities

There have been several activities that were necessary for the adequate functioning of the Directive, some were relevant in the early stage of the implementation, others are ongoing activities. The following are the key activities grouped according to the relevant actors.

Member States:

- Transpose the Directive into national law by Oct 2013 (i.e. to set up provisions regarding prior authorisation, where decided necessary; reimbursement of healthcare provided in another Member State under certain conditions; procedures and systems to be used in case of harm caused; continuity of healthcare, etc.);
- Set up and operate national contact points (NCP);
- Provide mutual assistance to each other and facilitate cooperation in cross-border healthcare provision at regional and local level through ICT and other forms.

National Contact Points:

- Make information available to citizens about cross-border healthcare that is discoverable, accessible and comprehensible;
- Consult with patient organisations, healthcare insurers and healthcare providers.

Key stakeholders:

- Health insurance bodies make decisions on prior authorisation (where applicable) and reimburse costs of cross-border healthcare;
- Patient organisations raise awareness to legal rights;
- Healthcare providers provide required medical information to patients.

European Commission:

- Facilitate coordination[9] and information exchange across Member States through the Cross-Border Healthcare Committee and its Expert Group;[10] that supports cross-border procedures in different legal areas, including Patients' rights in cross-border healthcare, e.g. information provided across the EU about countries blacklisting their providers based on safety and quality of services);
- Help EU citizens understand their rights to cross-border healthcare through various EU platforms (e.g. Your Europe Advice, SOLVIT);
- Monitor and assess operations of the Directive through conducting annual survey in Member States and draw up a report every three years.

2.6 Outputs

The outputs are considered the direct and attributable results of the Directive that may be measured or assessed if needed.

- Legal certainty for EU citizens about their rights, but also legal certainty about obligations for health care providers & social security institutions
- Functioning NCPs established in all Member States providing information to patients;
- EU citizens are aware about their rights and entitlements;

[9] Directive (49): "The Commission should work together with the Member States in order to facilitate cooperation regarding national contact points for cross-border healthcare, including making relevant information available at Union level."

[10] Directive Art. 10(4): "The exchange of information shall take place via the Internal Market Information system". See https://ec.europa.eu/internal_market/imi-net/library/index_en.htm. Administrative cooperation: areas and legal bases. Patients' rights. Information requests. To check a health professional's "right to practise".

- Member States cooperate on healthcare particularly in border regions. Bilateral agreements signed between Member States, particularly between neighbouring countries, on cross-border healthcare provision[11];
- Enhanced transparency and comparability of healthcare on safety, quality, cost and waiting times across the EU. While this was not foreseen as an aim, it was probably an aspiration of the Directive so that patients can make informed choice about cross-border healthcare.[12]

2.7 Outcomes

Linked to the outputs of the Directive, there are a number of outcomes one can reasonably expect from the implementation and functioning of the Directive (i.e. once the Directive is transposed, NCPs are set up and relevant information is available to citizens,):

- Patients seek cross-border healthcare in another EU country after making informed choices about their treatment options (i.e. freedom of movement of patients);
- Patients have earlier access to safe and high-quality healthcare abroad, for example for services that may already be available in the Member State of affiliation but where patients may experience long waiting times and undue delays;
- Patients are (partially) reimbursed by their health insurer for the cross-border healthcare costs incurred in accordance with the provisions set out in the Directive;
- Patients are provided continuity of care after specific treatments were provided by healthcare providers abroad, including through the systematic provision of medical record of treatment to patients, or mutual recognition of prescriptions issued in another Member State. Continuity of care also requires these foreign medical records to be integrated into the domestic health system;
- Enhanced cooperation on healthcare (in particular in border regions) and more efficient use of health system capacity and resources in Member States and across the EU;
- Learning and take up of good practices across national health systems, as a result of enhanced transparency and comparability of healthcare across the EU.

Finally, in the longer term, the Directive is expected to contribute to the following aspects:

- Improved health outcomes and patient experience for EU citizens, including improved health literacy and empowerment of patients;
- Overarching values of universality[13], access, equity and solidarity are ensured in the EU;
- For particular cross-border regions:

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[11] Directive Art. 10(3): "The Commission shall encourage Member States, particularly neighbouring countries, to conclude agreements among themselves. The Commission shall also encourage the Member States to cooperate in cross-border healthcare provision in border regions."

[12] This information about public healthcare providers remains closely linked to the national healthcare system and often not (publicly) available; private providers also do not routinely make such information available. A possible indirect effect of NCPs providing information on healthcare services to foreign citizens is that transparency on healthcare provision to domestic patients is also enhanced. This paradoxically may lead to reduced patient flows cross-border under the Directive. However, improvement of national social security systems is out of scope of the Directive as it aims to establish rules whilst "fully respecting the responsibilities of the Member States for the definition of social security benefits relating to health and for the organisation and delivery of healthcare and medical care and social security benefits".

[13] Note the Council Conclusions on Common values and principles in European Union Health systems (OJ C 146, 22.6.2006, p. 1–3).

- Enhanced EU cohesion, economic growth and competitiveness due to a healthier workforce;
- Reduced environmental footprint are linked to healthcare services as a result of patients travelling to the closest specialist/hospital across the border.

2.8 External factors

The effects of the Directive may be enhanced or attenuated due to external and more contextual factors[14]:

- Language barriers between patients, NCPs and healthcare staff/ administration
- Differing level of healthcare costs in Member States;
- Differing ways of prescribing, authorising and invoicing for healthcare services, including reimbursement status of medicinal products, medical devices (e.g. knee brace) and medical services (e.g. physiotherapy);
- Differing level of information available about and practices in public and private healthcare providers;
- Third way to access cross-border healthcare (beyond Regulation 883/2004 and Directive 2011/24/EU): Parallel cross-border healthcare agreements between Member States (bilateral and multi-lateral agreements)[15];
- Regulation 883/2004 on the coordination of social security schemes;
- Directive 2005/36/EC on mutual recognition of professional qualifications. Note however that tasks that can be carried out by professionals of the same qualification may differ from Member State to the next (e.g. who can inject? who can prescribe? Doctors versus nurses);
- Regulation 2016/679 on General Data Protection (GDPR);
- European Civil Protection Mechanism;
- EU Health Programme / Research Framework Programmes;
- eHealth Digital Service Infrastructure for data exchange on ePrescription and patient summaries;
- Lifestyle changes may involve increased travels and stays of citizens in other Member State than Member State of affiliation (i.e. retirees, seasonal workers)[16].\

2.9 Assumptions

Finally, there are general assumptions that would need to hold so that result of the various activities can lead to the expected impacts:

- Member States enforce national legislation around patient rights and implement it in the spirit of the Directive;
- Member States pro-actively inform EU citizens about their rights to a high-quality, safe healthcare and cost of the treatment in another EU country;
- Member States apply the Directive to all forms of healthcare, including medicinal products and medical devices;

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[14] For further information, see Annex 4.2 Thematic List of Council of Europe Treaties relating to Patient Rights, Patients' Rights in the European Union, Mapping eXercise, Final Report, PRE-MAX Consortium (2018).

[15] See for example specific bilateral framework agreements signed by France with Germany, Belgium, Spain, Luxembourg, Switzerland. https://www.cleiss.fr/docs/cooperation/index_en.html.

[16] To note that seasonal workers are mentioned in 2012 Impact assessment (recognition of medical prescriptions); the intended measures are expected to benefit the movement of specific groups of citizens (with particular chronic diseases, allergies, etc.) and services (e.g. short-term posted workers abroad).

- Stakeholders (including healthcare providers) in the health ecosystem cooperate to ensure continuity of care and timely reimbursement;
- Healthcare providers (including private sector) invoice foreign patient at the same level of fees as domestic patients;
- Health insurers reimburse healthcare up to the same level of treatment costs at home;
- Member States (uniformly) collect data related to cross-border patient flows to enable monitoring, assessment & learning.

2.10 Indicators for evaluation purposes

Data on the patient flows has systematically been collected each year since 2015 with a questionnaire being sent to all Member States, Norway and Iceland. Data collected in these questionnaires is meant to provide input for indicators needed to assess the **functioning** of the Directive (article 20). The results are summarised and published in annual data reports. The questionnaire used for data collection consists of 5 sections regarding: requests received by the National Contact Points, limitations to patient inflow, prior-authorisation data (requests, authorisations and refusals), non-prior-authorisation data (requests, authorisations and refusals), and free text on any issue for respondents to fill in. However, as pointed out in the most recently published 2019 annual data report, there have been limitations in terms of data collection, with many Member States only being able to provide partially completed questionnaires.

In addition to the annual data reports, the Commission was first required to submit a report on the **operation** of the Directive in 2015, with a second report submitted in 2018 and every 3 years thereafter. These reports are required to provide information on patient flows, financial dimensions of patient mobility, the implementation of Article 7(9) and Article 8 and on the functioning of the European Reference Networks, HTA network, eHealth network, and NCPs. In the Commission's 2015 report[17] a total of 26 Member States (out of a total 28 Member States at the time) provided information. However, the report did not cover complaints, infringements and transposition measures (these were not part of its remit). These may be important indicators to collect data for future monitoring and evaluation. The second and most recent report on the operation of the Directive was conducted in 2018.

This study aims to build on these reports and existing body of evidence from the literature review and identify a mix of quantitative and qualitative indicators that may be used in the first instance for the forthcoming **evaluation** of the Directive. These are linked to the Directive's intervention logic and structured along the standard evaluation criteria of **Effectiveness, Efficiency, Relevance, Coherence and EU added value.**

In the following sections, the final set of qualitative indicators and quantitative indicators are provided along with the potential data sources for the different evaluation criteria. These were assessed and discussed where possible during the stakeholder workshop. A summary of the workshop discussions are provided below.

[17] Commission Report on the operation of Directive 2011/24/EU on the application of patients' rights in cross-border healthcare.

2.10.1 Effectiveness

Indicators in this section consider how successful the Directive and associated activities have been in achieving (or progressing) toward its objectives. Therefore, effectiveness indicators should measure the effects of the Directive.

Qualitative indicators	Quantitative indicators	Data sources
1. Perception on clarity of responsibilities regarding cross-border healthcare; 2. Perception on clarity of reimbursement rules on cross-border healthcare costs; 3. Perception on extent and clarity of information provision by NCPs (rights and entitlement).	1. Number of incoming and outgoing patients per EU Member State per year (per treatment category if available); 2. Number of people reimbursed for healthcare provided in another Member State; 3. Number of prior-authorisation procedures versus non-prior-authorisation procedures (received, refused and authorised requests); 4. Number of prior notifications (where implemented) on the amount to be reimbursed and the cost of treatment; 5. Aggregate amount (in Euros) reimbursed by each country (for healthcare with and without prior authorisation).	<u>Qualitative</u> • Interviews; • Targeted survey (patient organisations, HCPs, healthcare insurance bodies, NCPs); • Case study; • Eurobarometer data; • Commission on-line public consultation. <u>Quantitative</u> • Annual data reports; • National document review; • Targeted survey (NCPs/health insurers); • Data request from ombudsman, court, ministries of health on patient complaints; • Patient registries; • National guidelines.

2.10.2 Efficiency

Indicators in the efficiency section consider the relationship between the level of inputs (costs and resources) available to the Directive compared to the outputs and benefits generated to different stakeholders.

Qualitative indicators	Quantitative indicators	Data sources
1. Perception of administrative burden on patients, HCPs and healthcare insurance bodies (Burden should be defined as additional to national situations).	1. Administrative costs for handling applications for prior authorisation, and reimbursement (incl. translation costs, assimilation to health system and calculation of amount to be reimbursed);	<u>Qualitative</u> Interviews (HCPs, healthcare insurance bodies, NCPs) Targeted survey Commission on-line public consultation <u>Quantitative</u> Targeted survey (Health insurance funds, NCPs)

Qualitative indicators	Quantitative indicators	Data sources
	2. Administrative waiting times to process requests for prior authorisation; 3. Administrative waiting times to process requests for reimbursement; 4. Number of patient complaints about administrative procedures.	Annual data reports

2.10.3Relevance

Indicators of relevance look at the changes in the needs identified during the impact assessment and current needs and their relationship to the Directive's stated objectives.

Qualitative indicators	Quantitative indicators	Data sources
1. Perception on the current and future needs of EU citizens for cross-border healthcare; 2. Perceptions on technological developments with implications on activities delivered by NCPs, HCPs, health insurance bodies; 3. Perceptions on technological developments with implications on Member State cooperation for cross-border healthcare.	1. N/A	Qualitative • Interviews (HCPs, healthcare insurance bodies, NCPs); • Targeted survey; • Commission on-line public consultation.

2.10.4Coherence

Indicators in this section consider synergies of the various articles and associated actions enabled by the Directive (internal coherence) that have an impact on its ultimate performance. Similarly, coherence shall look at how the Directive is consistent with actions of related pieces of legislations related to cross-border healthcare.

Qualitative indicators	Quantitative indicators	Data sources
1. Perceived clarity of the relationship between the Directive and the Social Security Coordination Regulation.	1. Number of legal processes initiated about administrative procedures.	Qualitative • Interviews (healthcare insurance bodies, NCPs); • Targeted survey.

Qualitative indicators	Quantitative indicators	Data sources
		Quantitative • Data request from ombudsman, court, ministries of health on patient complaints.

2.10.5 EU added value

Finally, indicators of added value at the EU level consider those changes that can be attributed to the EU intervention which is beyond any national action only.

Qualitative indicators	Quantitative indicators	Data sources
1. Perceived benefit of support provided by the EU to patients with regard to cross-border healthcare services 2. Perceived benefit of support provided by the EU to Member States with regard to cross-border healthcare cooperation	1. N/A	Qualitative • Interviews (healthcare insurance bodies, NCPs, Ministries of Health); • Targeted survey; • Case study.

2.11 Summary of workshop discussion – Patient rights

In attendance at the workshop were 35 stakeholders that covered NCPs and representatives of Ministries of Health, health insurance bodies and healthcare providers, as well as Commission staff as observers. However, representatives of patient organisations did not attend the breakout room on patient rights therefore their perspective may not be fully reflected in the discussion. The list of registered organisations is provided in 0.

After the presentation of the Intervention Logic of the Directive that was developed as part of the study, an interactive session on providing feedback on the longlist of indicators followed. Stakeholders were informed that qualitative indicators are collected as primary consultation with stakeholders either bespoke surveys (targeted, the Commission's online public consultation or Eurobarometer) or interviews with stakeholders during the evaluation. Quantitative indicators are on the other hand rely on data already collected and available via various stakeholders in Member States.

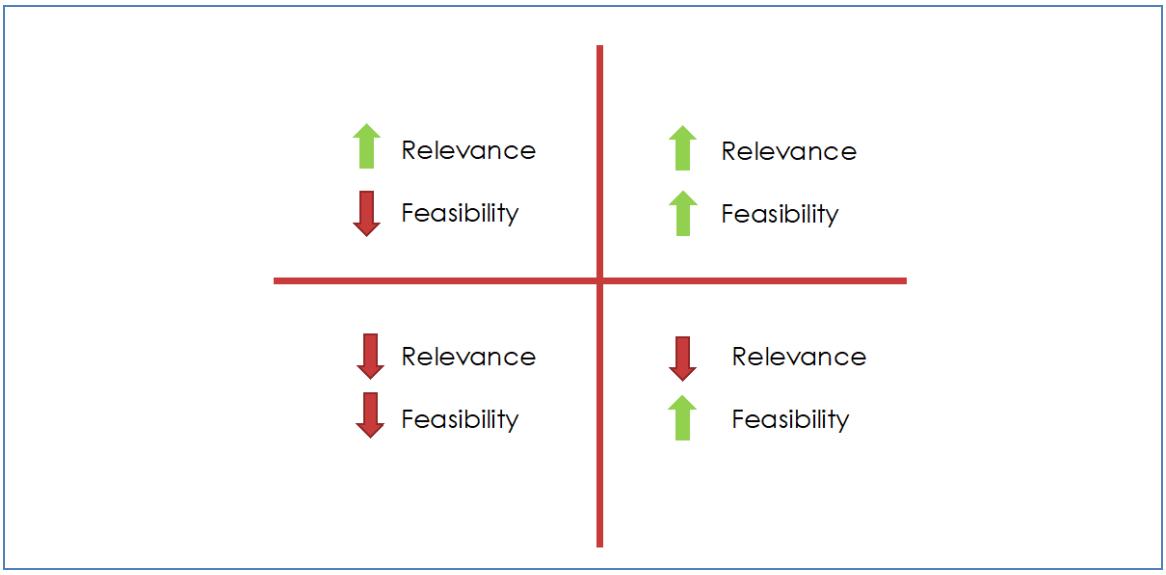
Workshop participants were asked to vote on the relevance (high relevance or low relevance) and feasibility (high feasibility or low feasibility) of each indicator presented. In cases, where indicators were deemed of low relevance, indicators were not further discussed. Shortlisted indicators were those that were voted to have been both highly relevant and highly feasible to collect data for (upper right quadrant in Figure 2.2). Some indicators, while deemed highly relevant, were of low feasibility and discussion was encouraged on these indicators (upper left quadrant). The discussions with stakeholders aimed to understand data collection challenges and to assess whether these indicators may be shortlisted (perhaps with limitations) for the forthcoming

evaluation. In particular, the study team has put forward the following questions to drive the discussions:

- Who currently holds the data?
- Is baseline data before 2011 available?
- Is there an agreed target for these indicators?
- Is it feasible for small/big countries?
- Is it feasible in (de)centralised systems?
- Could indicators be made more feasible?

However, the time available for detailed discussions was limited but valuable comments and feedback in the online forum have been systematically collected and used in the preparation of this final analytical report. The independent study team ultimately made the suggestion if indicators with low feasibility are included in the shortlist.

Figure 2.2 Indicators were grouped according to relevance / feasibility criteria



Source: Technopolis 2021.

2.11.1 Effectiveness indicators

A large majority of participants agreed that **qualitative indicators** on perceptions on the clarity of responsibilities, reimbursement roles, and information provision by NCPs are all highly relevant and highly feasible to collect. On the other hand, a number of qualitative indicators were deemed unfeasible to collect: extent and clarity of information provision by healthcare professionals, continuity of care, change in cooperation by Member States on cross-border healthcare provision, and the change in comparability of safety and quality of healthcare across the EU (which would enhance transparency and comparability of healthcare in the EU). It is nevertheless information that may be provided by patients and their representatives.

The top (in terms of relevance and feasibility) **quantitative indicators for effectiveness** were (i) Number of prior-authorisation procedures versus non-prior-authorisation procedures (broken down to received, refused and authorised requests); number of prior notifications (where implemented) on the amount to be reimbursed and the cost of treatment; aggregate amount reimbursed by each country (for healthcare

with and without prior authorisation). These "raw data" are reported in the annual data report by Member States with some exceptions.

Further indicators were shortlisted where although feasibility concerns were raised, relevance of these indicators are high: (i) Number of incoming and outgoing patients per EU Member State per year; (ii) Number of people reimbursed for healthcare provided in another Member State. These data are also reported in the Commission's annual data report but reporting issues arise and are explained in these Commission reports. More specifically, participants explained that in some countries (e.g. Austria, the Netherlands) reimbursement from insured persons who receive cross-border health treatments that do not require prior approval are treated like domestic reimbursement claims and are therefore not specifically recorded. Only cases in which prior authorisation did not take place due to a medical emergency during a temporary stay abroad are recorded separately. In addition, distinction in reporting data on cross-border healthcare between the Directive and the Regulation may not be identified (e.g., France).

Other indicators that were on the longlist were deemed of low relevance and unfeasible to collect: (i) Number of new bilateral agreements between Member States attributable to the Directive; (ii) Number of cross-border patient complaints about healthcare providers; (iii) Cross-border patient reported outcomes; and (iv) Change in distance travelled to cross-border healthcare facility by patients.

2.11.2 Efficiency indicators

Efficiency indicators cover costs and benefits of the system. While the various costs are usually paid upfront, benefits accrue over many years and often more challenging to monetise. Nevertheless, one qualitative and a number of quantitative indicators were shortlisted.

Qualitative indicator retained is the perception of administrative burden on patients, HCPs and healthcare insurance bodies. Stakeholders commented that administrative burden should be defined as additional to national situations. If specific extra tasks/actions are defined per case, an estimate can be made on average costs. This indicator has been updated accordingly.

Quantitative indicators include administrative waiting times to process (i.e. processing times) requests for (i) prior authorisation and (ii) reimbursements. These data are also reported in the Commission's annual data report but reporting issues arise and are explained in these Commission reports.

Other quantitative indicators had lower feasibility but due to their high relevance, we suggest including those in the longlist. The first is the administrative costs for handling applications for prior authorisation, and reimbursement. A workshop participant explained that healthcare professionals and insurers usually have an approximate average FTE resource apportioned to processing a particular patient cohort or business operation. These kinds of quantifications are normally used for general service planning and resourcing and should be available for the cost benefit analysis.

The second indicator with somewhat lower feasibility to gather data on is the number of patient complaints about administrative procedures. Workshop participants added that Member States are required to have an official complaints and appeal procedure in place and in some Member States a centralised record of complaints received. It was suggested that while many patients access cross-border healthcare through private providers that do not report directly to NCPs, insurance bodies or Ombudsman Offices,

patient complaint information should be available about both public and private providers (possibly after assurance of adequate handing of sensitive information at the right level of aggregation). However, further investigation into patient complaints procedures as a data source is necessary to check the feasibility of this indicator about the effectiveness of administrative procedures in the context of cross-border healthcare.

2.11.3Relevance indicators

Relevance indicators are all qualitative in nature and workshop participants suggested that it would be harder to collect data on such aspects. Nevertheless the study team suggested to retain these on the shortlist as in their experience stakeholders can provide such information through survey and interviews. One example is the perception on the current and future needs of EU citizens for cross-border healthcare. While it was suggested in the workshop to use the Eurobarometer survey, it was also suggested that such questions can be part of the Commission's online public consultation. Since patient organisations were not represented in the workshop, the public consultation can provide representative bodies the opportunity to contribute.

2.11.4Coherence indicators

Coherence indicators include one quantitative and one qualitative indicator. Quantitative indicator is about the number of legal processes initiated about administrative procedures. This information while may not be available from all Member States, a representative selection or case studies can provide insight into trends. Qualitative indicator to collect for coherence is the perceived clarity of the relationship between the Directive and the Social Security Coordination Regulation.

2.11.5EU added value indicators

Two qualitative indicators are proposed to measure the Directive's EU added value about perceived benefit of support provided by the EU to (i) patients and (ii) Member States with regard to cross-border healthcare services. For the latter, useful data source is the Commission study on projects on cross-border healthcare cooperation in 2018.[18] DG REGIO have also useful information about investment in Member States. In both cases however but the evaluation would need assess the attributable effect of the Directive on cross-border healthcare.

[18] https://ec.europa.eu/health/sites/default/files/cross_border_care/docs/2018_crossbordercooperation_frep_en.pdf.

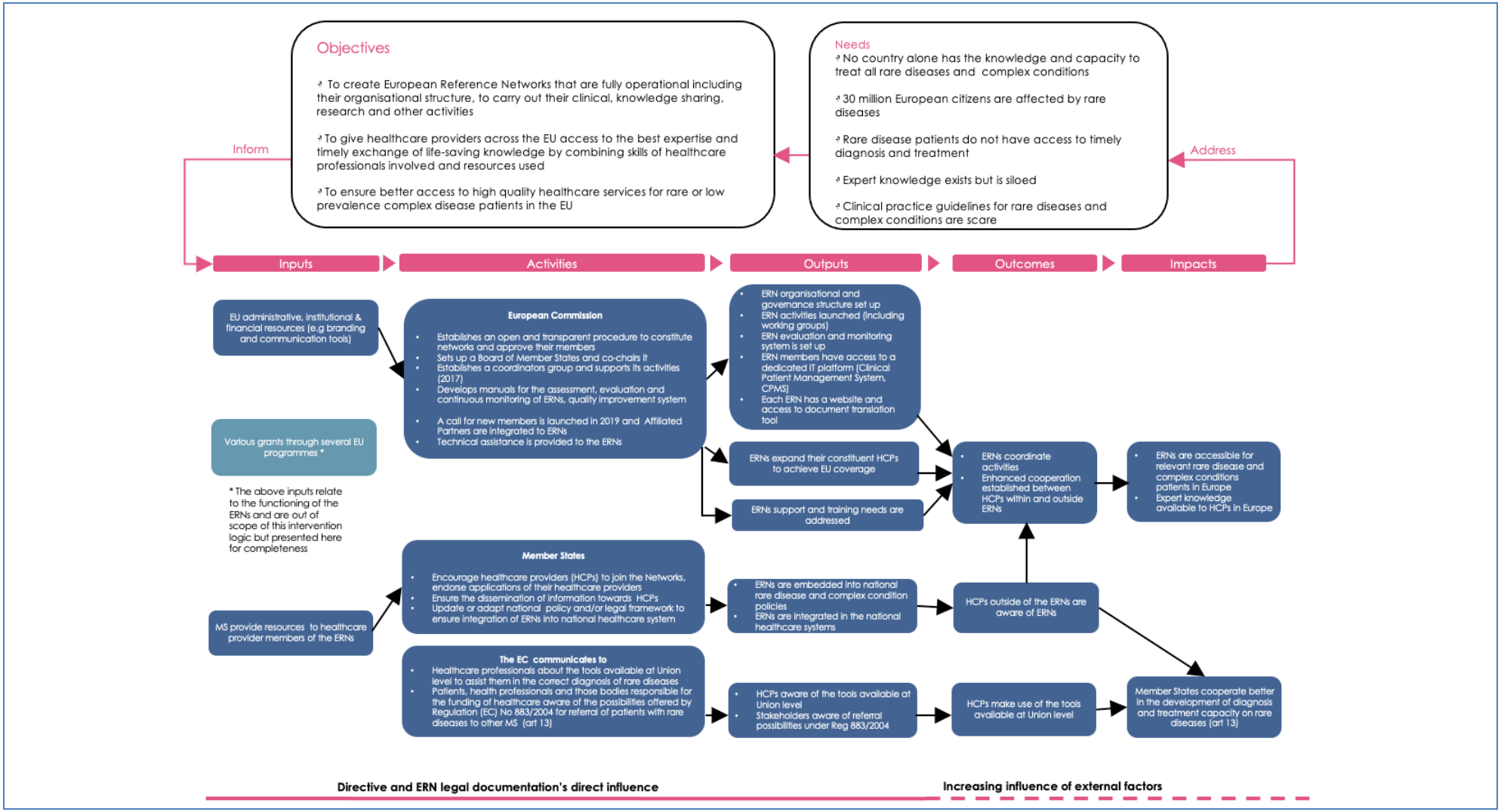
3 Rare diseases and the European Reference Networks

3.1 Introduction

The ERNs are virtual networks that involve healthcare providers (HCPs) across Europe to help treat complex or rare diseases and conditions. The following section describes the concept of an intervention logic followed by a pictorial representation of the draft intervention logic model developed for the establishment of the European Reference Networks (ERNs), based on a literature review of key documentation and targeted interviews. Further descriptive exploration of each section of the intervention logic is provided subsequently. Potential indicators for evaluating the ERN policy concept are then explored, with a list of qualitative indicators suggested where data gaps have been identified. Lastly, we provide next steps, which involves a stakeholder workshop to finalise the material presented here.

Note that the scope of this study and hence the intervention logic does not encompass the entire causal chain of the intervention but is limited to **how the ERNs were setup, focusing on aspects of the policy framework and governance** rather than actual implementation. A separate study preparing the future performance review of ERNs is ongoing. The partial intervention logic developed in this work was conducted retrospectively and includes elements that were not foreseen specifically in the Directive but that were deemed important to understand how the setup of the ERNs may lead to their effective functioning.

Figure 3.1 Intervention logic of the setup of the European Reference Networks (Directive 2011/24/EU Articles 12-13)



Source: Technopolis Group 2021.

3.2 Needs

- No country alone has the knowledge and capacity to treat all rare diseases and complex conditions[19];
- 30 million European citizens are affected by rare diseases[20];
- Rare disease patients do not have access to timely diagnosis and treatment;
- Expert knowledge exists but is siloed;
- Clinical practice guidelines for rare diseases and complex conditions are scare[21].

Rare diseases and complex conditions often require highly specialised treatment and knowledge. While the prevalence of an individual rare disease is not insignificant, overall 30 million European citizens are affected by thousands of different rare diseases. These patients often do not have access to a timely diagnosis of their condition. There are also too few clinical practice guidelines available to healthcare providers for rare diseases and complex conditions. Moreover, the resources needed for treatment of all forms of rare disease[22], in terms of personnel, infrastructure and finance cannot be feasibly provided in the bounds of national or regional healthcare systems.[23] More likely is that there will be clusters of expertise and resources to treat certain rare diseases but be less able to treat other rare diseases. It is therefore important to create a networks of specialist healthcare providers, pool EU expertise, develop best practice and share knowledge in rare diseases across Member States, providing all rare disease patients with access to the best possible diagnosis and treatments.

3.3 Objectives

High level objectives:

1. To create ERNs that are fully operational including their organisational structure, to carry out their clinical, knowledge sharing, research, and other activities;
2. To give healthcare providers across the EU access to the best expertise and timely exchange of life-saving knowledge by combining skills of healthcare professionals involved and resources used[24];
3. To ensure that EU patients have better access to high quality healthcare services for rare or low prevalence complex disease [25].

Specific objectives:

1. To improve access to virtual clinical advice, diagnosis, treatment and follow-up of rare disease and complex conditions patients, across geographies and diseases, reducing the need for physical travel [26], [27];

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19 ERN 2017 Brochure https://ec.europa.eu/health/sites/health/files/ern/docs/2017_brochure_en.pdf pg 6.
20 ERN Conference Report 2018 https://ec.europa.eu/health/sites/health/files/ern/docs/ev_20181121_frep_en.pdf Pg 4.
21 Pavan et al 2017 Clinical Practice Guidelines for Rare Diseases: The Orphanet Database.
22 IBID.
23 https://ec.europa.eu/health/sites/health/files/ern/docs/2017_brochure_en.pdf pg 6.
24 IBID.
25 IBID.
26 Commission Delegated Decision of 10 March 2014 Pg 1 https://ec.europa.eu/health/sites/health/files/ern/docs/ern_delegateddecision_20140310_en.pdf.
27 Continuous Monitoring of ERNs: https://ec.europa.eu/health/sites/health/files/ern/docs/continuous_monitoring_en.pdf Pg 6.

2. To reinforce clinical research in the field of rare diseases and complex conditions by collecting data and carrying out collaborative research activities[28];
3. 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 in the EU[29];
4. To guarantee that knowledge and expertise is spread outside the ERN so that more patients and health professionals can benefit from the ERN activities.[30]

The objectives of the ERNs are set out in the ERNs' legal framework[31],[32],[33],[34] and are also further categorised by the ERN Continuous Monitoring Working Group of the ERN Coordinators Group and the Board of Member States.[35] The objectives have been organised here into 3 High level and 4 Specific objectives.

The first high level objective of the ERNs is to ensure that each network is operational, including its organisational structure, and that each can carry out its clinical, knowledge sharing, research and other activities. Organisational activities for each network include ensuring that they are represented by enough stakeholders to function. These stakeholders may be healthcare providers (HCPs), affiliated partners and patient organisations.

The second high level objective states that HCPs across the EU have access to the best expertise and timely exchange of life-saving knowledge. Through the operationalising of the ERNs HCPs gain access to pool of expertise in rare diseases and complex conditions that they otherwise may not have.

Given that the ERNs have ultimately been set up to help patients with rare diseases and complex conditions, the third high level objective concerns better patient access to high quality care.

Specific objective 4 relates to High level objective 3, however goes into more detail as to how patients may stand to benefit (via virtual clinical advice, diagnosis, treatment, and follow-ups).

Specific objective 5 leads on from High-level objective 1 and seeks to reinforce clinical research in the field of rare diseases and complex conditions by collecting data and carrying out collaborative research activities. Clinical research may include the development of new best practice guidelines, new randomised clinical trials or observational studies. The need, particularly for new trials is clear, the majority of the roughly 7,000 rare diseases still lack a specific treatment.[36] Expert capacity and knowledge, either pooled from existing experts (Specific objective 6) or generated from new clinical studies through the ERNs can be used to create new best practice guidelines.

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28 Continuous Monitoring of ERNs: https://ec.europa.eu/health/sites/health/files/ern/docs/continuous_monitoring_en.pdf Pg 6.
29 IBID.
30 IBID.
31 DIRECTIVE 2011/24/EU https://eur-lex.europa.eu/eli/dir/2011/24/oj.
32 Commission Delegated Decision of 10 March 2014 https://eur-lex.europa.eu/legal-content/EN/TXT/?uri=CELEX%3A32014D0286.
33 2014/287/EU: Commission Implementing Decision of 10 March 2014 https://eur-lex.europa.eu/legal-content/EN/TXT/?uri=OJ%3AJOL_2014_147_R_0007.
34 Commission Implementing Decision (EU) 2019/1269 of 26 July 2019 amending Implementing Decision 2014/287/EU https://eur-lex.europa.eu/legal-content/EN/TXT/?uri=CELEX%3A32019D1269.
35 Continuous Monitoring of ERNs: https://ec.europa.eu/health/sites/health/files/ern/docs/continuous_monitoring_en.pdf.
36 Tambuyzer et al (2020) Therapies for rare diseases: therapeutic modalities, progress and challenges ahead.

In turn, this new knowledge can then be disseminated to clinicians outside of the ERNs (Specific objective 7).

Specific objective 6 (relating to High level objective 2) is to increase capacity of professionals to recognize and manage cases of rare or low prevalence complex diseases. This objective is included in recognition of the need to provide education, training and capacity of health professionals to treat rare diseases. Examples of education activities are the organisation of Continuing Medical Education (CME) courses and hosting webinars for professionals. The European Rare Kidney Disease Reference Network (ERKNet), for example, runs webinars every two weeks which attract 100-150 people per live session (and an additional 150 - 200 downloads of the recorded event).[37]

Specific objective 7 is similar to Specific objective 6 in that it regards knowledge dissemination, however, it differs in that the knowledge should be disseminated outside of the ERNs to those who may benefit, rather than to clinical professionals who operate within the ERNs.

3.4 Inputs

Inputs for the establishment of the networks

1. European Commission (EC) sets up ERN secretariat to support the ERN Board of Member States, the ERN Coordinators' Group and their working subgroups and organise members' expertise and resources;
2. EC provides resources for information exchange and communication on the Networks:
    - EC ensures consistent ERN branding by providing communication and digital support for documentation (document templates, logo design and a newsletter) and communication tools to the ERNs (including websites);
    - EC organises conferences and expert meetings.
3. EC provides the expertise and resources required for virtual clinical consultations
    - EC helps to set up, use, maintain and improve the Clinical Patient Management System (CPMS), an ERN IT tool for virtual consultations between health professionals.
4. Member States provide resources to healthcare provider members of the ERNs according to rules of their national health system.
5. Funding per ERN is available for network coordination over a five-year period[38]
    - ERNs receive support from several EU programmes, such as the Health Programme, the Connecting Europe Facility, Horizon 2020 and the European Joint Programme on Rare Diseases[39], which are essential for their functioning (out of scope for present work).

The Art. 12 of the Directive states that the EC is to support the Member States in the development of the European Reference Networks. In order to deliver on the objectives of the ERNs, the EC and Member States engage resources of different kinds. The EC

[37] https://ec.europa.eu/health/sites/health/files/ern/docs/ev_20181121_frep_en.pdf.
[38] ERN Conference 2018 pg 5 https://ec.europa.eu/health/sites/health/files/ern/docs/ev_20181121_frep_en.pdf.
[39] Note the EJP RD is co-funded by Horizon Europe (55 M€) and Member States (46 M€).

provides inputs in the form of financial support to the ERNs and operational support such as the development of a bespoke IT system and communication tools.

Member States provide resources to the ERN members among their healthcare providers.

ERNs receive financial support from the EC but also have access to support from a variety of EU programmes. This aspect relates to inputs for the running of the ERNs activities, which does not fall specifically under the scope of this intervention logic, however they are an important element to understand ERNs are supported through various channels (for example beside training activities organised by the ERNs themselves and/or supported by the EU Health programme, ERNs could also benefit from training activities related to rare disease research within the European Joint Programme on Rare Diseases (EJP RD).

3.5 Activities

Activities for the establishment of the networks

1. The European Commission:
    - establishes an open and transparent procedure to constitute networks and approve their members;
    - sets up a Board of Member States *(to approve Networks, membership and termination and to take strategic decisions[40])* and co-chairs it with an elected representative of a MS;
    - establishes a coordinators group and supports its activities (2017);
    - adoption of the Implementing and Delegated Decisions by the Commission;
    - develops manuals for the assessment, evaluation and continuous monitoring of ERNs, quality improvement system;
    - provides technical and financial assistance to ERNs to support their activities (e.g. for the development, appraisal and implementation of Clinical Practice Guidelines (CPGs) and Clinical Decision Support Tools (CDSTs));
    - launched a call for new members in 2019 and integrated Affiliated Partners to enlarge the ERNs.
2. Member States:
    - encourage healthcare providers to join the Networks, endorse applications of their healthcare providers[41];
    - ensure the dissemination of information towards healthcare providers;[42]
    - update or adapt national policy and/or legal framework to ensure integration of ERNs into national healthcare system.

Activities relevant for Art 13

1. The EC communicates to:

[40] COMMISSION IMPLEMENTING DECISION of 10 March 2014 Pg 4. https://eur-lex.europa.eu/legal-content/GA/TXT/?uri=CELEX:32014D0287.
[41] COMMISSION IMPLEMENTING DECISION of 10 March 2014 Pg 2. https://eur-lex.europa.eu/legal-content/GA/TXT/?uri=CELEX:32014D0287.
[42] Article 12 (3) of the Directive.

- healthcare professionals of the tools available to them at Union level to assist them in the correct diagnosis of rare diseases;
- patients, health professionals and those bodies responsible for the funding of healthcare aware of the possibilities offered by Regulation (EC) No 883/2004 for referral of patients with rare diseases to other MS.

The establishment and functioning of ERNs are the result of activities run conjointly by the EC and Member States. On the one hand, the EC and Member States run activities to establish and ensure the ERNs can function properly. On the other hand, once established, the activities of the ERNs are run by the ERN Coordinators, project managers and ERN managers. The current intervention logic focuses on the establishment and governance of the networks.

The networks are established, and Members approved on the basis of an open and transparent procedure[43], as follows:

- EC to publish a call for interest to establish ERNs within 2 years of implementing decision[44];
- EC to appoint an Independent assessment body[45] and to draw up a detailed manual regarding the content of, documentation and procedure for the assessment of membership applications;
- Potential network members and networks apply to a Call from the EC, proposals are technically assessed and approved by the EC, independent assessment body, Board of the Member States and (after amendment in 2019) by the boards of the networks (see the Implementing Decision, Articles 9 and 10)[46];
- Each Network is to select at least three objectives from the list laid down in Directive 2011/24/EU and demonstrate that it has the necessary competences to pursue them effectively[47];
- Members comply with the list of criteria and conditions for potential network members drafted by the EC to become Member of a Network[48];
- Each Network appoints a coordinator and is governed by a Board of the network which pilots the activities of the Network[49].

Moreover, in 2017, the EC established a coordinators group to plan cooperation among networks and to decide on issues of common interest for all networks – including one on integration to national healthcare system. The coordinators group meets twice a year in a meeting arranged by the EC. This group, together with the Board of the Member States (BoMS), has set up 7 ERN working groups on key areas for ERNs to collaborate effectively on, co-chaired by the EC (e.g. integration of ERNs in the national healthcare system of MS, ensuring representative members in each network). Recently, the

43 COMMISSION IMPLEMENTING DECISION of 10 March 2014 Pg 1. https://eur-lex.europa.eu/legal-content/GA/TXT/?uri=CELEX:32014D0287.
44 COMMISSION IMPLEMENTING DECISION of 10 March 2014 Pg 1. https://eur-lex.europa.eu/legal-content/GA/TXT/?uri=CELEX:32014D0287.
45 COMMISSION IMPLEMENTING DECISION of 10 March 2014 Pg 2. https://eur-lex.europa.eu/legal-content/GA/TXT/?uri=CELEX:32014D0287.
46 COMMISSION IMPLEMENTING DECISION of 10 March 2014 Pg 2. https://eur-lex.europa.eu/legal-content/GA/TXT/?uri=CELEX:32014D0287.
47 COMMISSION DELEGATED DECISION of 10 March 2014 Pg 1. https://ec.europa.eu/health/sites/health/files/ern/docs/ern_delegateddecision_20140310_en.pdf.
48 COMMISSION DELEGATED DECISION of 10 March 2014 Pg 1. https://ec.europa.eu/health/sites/health/files/ern/docs/ern_delegateddecision_20140310_en.pdf.
49 COMMISSION DELEGATED DECISION of 10 March 2014 Pg 1. https://ec.europa.eu/health/sites/health/files/ern/docs/ern_delegateddecision_20140310_en.pdf.

working groups became joint groups bringing together representatives of the coordinators and the BoMS, with the Commission providing the secretariat.

The EC also ensures framework conditions for the operation of the ERNs in the form of manuals for monitoring and evaluation of the ERNs.

Member States (voluntarily) participate to the establishment of the networks by adopting legislation/policies integrating the ERNs in their national healthcare system, encouraging their healthcare providers to apply as members of the networks and by ensuring their healthcare providers receive information related to the ERNs.

Under Article 13, the EC is engaged to raise awareness of healthcare professionals of the tools available to them for the diagnosis of rare diseases. The EC also makes patients, of healthcare professionals and those bodies responsible for the funding are of the possibilities offered by Regulation (EC) No 883/2004 for referral of patients with rare diseases to other MS.

3.6 Outputs

Outputs of the establishment of ERNs

1. ERN organisational and governance structure is set up (e.g. ERN managers are hired);
2. ERNs activities launched (including working groups);
3. ERN evaluation and monitoring system is set up;
4. ERN members have access to a dedicated IT platform (Clinical Patient Management System (CPMS));
5. Each ERN has a website and access to document translation tool;
6. ERNs support and training needs are addressed;
7. ERNs expanded their constituent healthcare providers to achieve EU coverage;
8. ERNs are embedded into national rare disease and complex conditions policies;
9. ERNs are integrated in the national healthcare systems.

Outputs for Art 13

1. Health professionals aware of the tools available at Union level;
2. Stakeholders aware of referral possibilities under Regulation 883/2004.

Outputs are the direct consequences of activities. The outputs listed above refer on the one hand to the practical organisation of the ERNs, and on the ongoing improvement or support provided for the ERN functioning and on the other hand to the governance aspect related to the MS activities.

The first set of outputs (1 to 5) concerns the building blocks for the operations of ERNs. The overall organisation of the networks is set up and their governance established on basis of the inputs and activities of both the EC and the MS. The networks launch their activities after being approved by the BoMS and have access to the tools they need to operate (e.g. CPMS platform, website, communication tools and translations).

The activities of the EC to provide ongoing support to the ERNs result in two outputs. The needs of the ERNs in terms of support and training are addressed in order for them to deliver their activities (output 6) and the ERNs networks expand the number of their members (output 7).

Finally, the activities of the Member States, but also the call for new members of 2019 lead to Member States participating actively to the ERNs, embedding the ERNs in their national rare disease and complex conditions policies but also integrating the networks in their national healthcare systems.

Under Article 13, the health professionals but also patients and bodies responsible for the funding of healthcare are aware of the tools available at Union level as well as the possibilities offered by Regulation (EC) No 883/2004 for referral of patients with rare diseases to other MS.

3.7 Outcomes

1. ERNs coordinate activities;
2. Enhanced cooperation established between healthcare providers within and outside ERNs;
3. HCPs outside of the ERNs are aware of ERNs.

Outcomes for Art 13

1. Healthcare professionals make use of the tools available at Union level.

Outcomes here are explored in relation to the Outputs, links between are provided where possible. Outcomes 1 and 2 are the direct result of the outputs 1-7.

The setting up and running of ERNs as well as the communication support allow for a better cooperation of healthcare providers but also supports the coordination of activities across ERNs, such as working groups. The ERNs activities and the improved cooperation are also facilitated by the ongoing support of the EC, notably through technical assistance contract leading to addressing the needs of the networks (e.g. in developing decision-making tools).

With the integration of ERNs in national healthcare systems and strategies (outputs 8 and 9) it is expected that healthcare professionals outside of the ERNs are aware of ERNs (outcome 3).

Under the Article 13, once the health professionals and other stakeholders are made aware of the other tools and regulation 883/2004, they are expected to make use of them in order to support the correct diagnosis of rare diseases.

3.8 Impacts

1. ERNs are accessible for relevant rare disease and complex conditions patients in Europe;
2. Expert knowledge is available to healthcare providers in Europe.

Impacts for Art 13

1. Member States cooperate better in the development of diagnosis and treatment capacity of rare diseases (art 13).

The impact suggested here is limited due to the scope of this study concerning the policy and legislative aspects of the ERNs. The impact of setting up the ERNs is to make the ERNs accessible for the relevant rare disease and complex conditions patients in Europe.

Setting up the ERNs is also an avenue for healthcare providers to have access to European experts in rare disease and complex conditions.

Other impacts, such as improved health outcomes for patients with rare diseases and complex conditions, are impacts that are realised from the correct functioning of the ERNs themselves, so are not covered here.

Under Article 13, with healthcare professionals making use of the tools available at Union level, specifically the Orphanet database and the ERNs but also through the use of the Regulation 883/2004, Member States effectively cooperation better.

3.9 External factors[50]

Legislative factors:

1. Pharmaceutical Strategy, under which the revision of the paediatric & orphan medicinal products legislation is foreseen;
2. Departure of the UK from the EU and thus from coordination and membership of ERNs;
3. Regulation 2016/679 on General Data Protection (GDPR) / processing of patients' data according to GDPR [51];
4. Interaction with pharmaceutical /medical industry and the issue of the absence of legal status of the ERNs;
5. New Clinical Trials Regulation (EU) No 536/2014 coming into effect by the end of 2021;[52]
6. European Health Data Space (EHDS) (future legislative proposal on a EHDS).

<u>EU research and funding programmes such as:</u>

1. European Joint Programme for Rare Diseases (EJP RD) – this is a co-fund under Horizon 2020 Research & Innovation Framework Programme;
2. EU Health Programme / Research Framework Programmes (and other EU funding programmes e.g. Connecting Europe Facility).

<u>Strategic factors:</u>

1. Health Digital Service Infrastructure (eHDSI);
2. European Health and Digital Executive Agency (HaDEA);
3. EU Data Strategy (health included in 1 of 12 items);
4. European Platform on Rare Disease Registration (EU RD Platform);
5. Patients' rights organisations;
6. Integration into national healthcare systems as well as existence and implementation of national rare disease plans in which ERNs can be embedded;
7. No reimbursement for cross-border virtual advice.

External factors are elements beyond the control of the intervention that may influence its effects. Three types of external factors have been identified. First and foremost, the legislative factors that can influence the running of the ERNs. These include the revision

[50] For further information, see Annex 4.2 Thematic List of Council of Europe Treaties relating to Patient Rights, Patients' Rights in the European Union, Mapping eXercise, Final Report, PRE-MAX Consortium (2018).

[51] COMMISSION DELEGATED DECISION of 10 March 2014 Pg 2. https://ec.europa.eu/health/sites/health/files/ern/docs/ern_delegateddecision_20140310_en.pdf.

[52] Clinical Trial Regulation, European Medicines Agency. https://www.ema.europa.eu/en/human-regulatory/research-development/clinical-trials/clinical-trial-regulation.

of existing legislation, new planned legislation (in the legislative pipeline, a new clinical trial directive is coming into effect at the end of 2021) but also the uncertainty associated with the Brexit. Brexit has affected the role and participation of UK healthcare providers in ERNs: the eUROGEN study suggests that 6 UK members discontinued their membership with eUROGEN at the beginning of 2021.[53]

Along these legislative factors are the existing EU research and funding programmes with also operate in relation with the ERNs. This is the case of the EJP RD which includes a number of ERN members. Other EU programmes also provide grants to ERNs members which has an effect on their activities and results.

Changes at the EU level, especially initiative addressing the need for enhanced cooperation such as the launch of the new Executive agency HaDEA, the planned project on European Health Data space or the eHealth Digital Service Infrastructure may bring changes relevant to the transfer of (digital) health data across Member States, and therefore to the ERNs activities. These infrastructures ensure patients' data are shared securely and for the right purposes. In the particular field of rare diseases, the European Platform on Rare Disease Registration (EU RD Platform) is an existing EU tool which "copes with the fragmentation of rare disease patients' data contained in hundreds of registries across Europe" by developing standards for data collection and centralising several databases on rare diseases on the European Rare Disease Registry Infrastructure[54] (ERDRI). Notably, the EU RD Platform is already working with ERNs and Pillar 2 (data, Virtual Platform) of EJP RD.

Representing the final beneficiary of the ERNs, patients' organisations have a keen interest in the rare disease networks. EURORDIS, the European rare diseases organisation – which ensures that patients' voice is heard and alliance representing 733 rare disease patient organisations in 64 countries – has established 24 European Patient Advocacy Groups (EPAGs), mirroring the themes of the ERNs. EURORDIS participates in activities of ERNs and their outcomes.

Finally, national factors may influence the work of the ERNs, namely the quality of the integration of the ERNs in the national system as well as reimbursement/support systems.

3.10 Assumptions

1. Healthcare providers share knowledge, expertise and good practice;
2. Healthcare providers adopt agreed data standards to facilitate rare disease health services across the EU;
3. Member States provide the necessary resources to develop infrastructures (including digital) and sustain activities;
4. Member States provide the legislative and financial support necessary to establish and run the ERNs.

There are assumptions regarding the participation of Member States and healthcare providers to ensure the success of the ERNs. First, the Member States participate through the BoMS which provides guidance and a general framework for the activities of the ERNs. This assumption supposes that MS are willing to engage healthcare

[53] Supplementary Appendix A Oomen, Loes, et al. "Rare and Complex Urology: Clinical Overview of ERN eUROGEN." European Urology (2021).
[54] https://eu-rd-platform.jrc.ec.europa.eu/erdri-description_en.

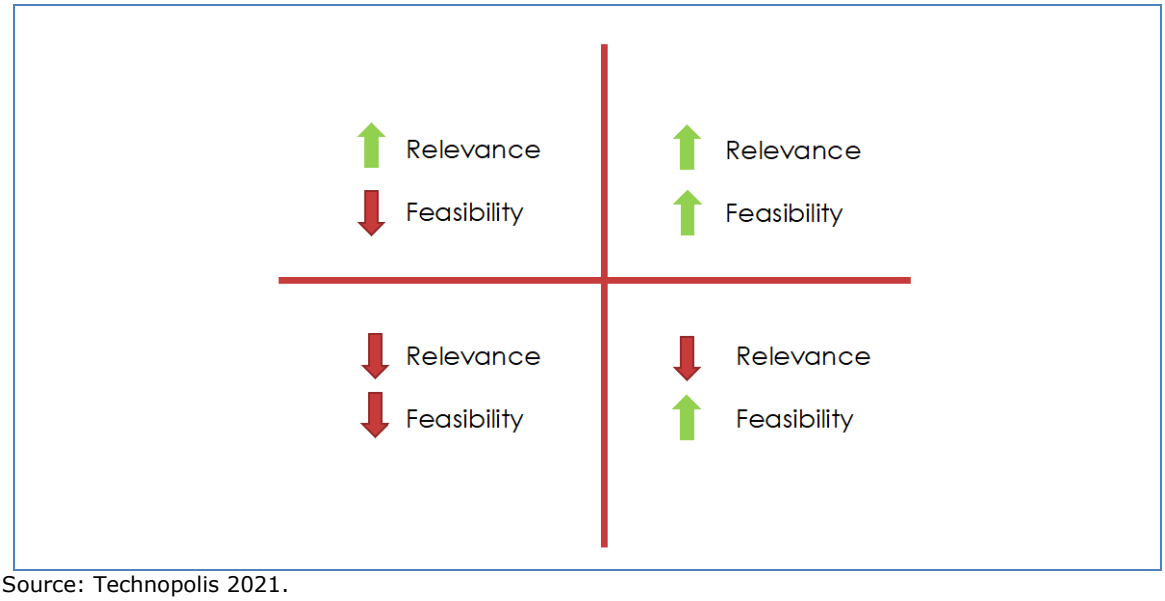
providers to apply as members (or associated national centres) to the networks, may designate a national coordination hub and make certain that ERNs are well connected to their national health services. Member States channel resources when participating to the BoMS (e.g., by certifying healthcare providers' membership application are in accordance with national legislation) but also by providing the legislative and financial support necessary to establish and run the ERNs. An important assumption is that Healthcare providers and Member States adopt health data standards to facilitate rare diseases health services across the EU.

3.11 Indicators

The current study aimed to build on available data and identify a **mix of quantitative and qualitative indicators** that may be used in the first instance for the forthcoming evaluation of the Directive to measure the progress that has been made on the ERNs. These were linked to the specific intervention logic and structured along the standard evaluations criteria of Effectiveness, Efficiency, Relevance, Coherence and EU added value.

In the following sections, a set of qualitative indicators and quantitative indicators are provided along with the potential data sources for the different evaluation criteria. Indicators presented in the tables below have been shortlisted after an interactive stakeholder workshop. Workshop participants were asked to vote on the relevance (high relevance or low relevance) and feasibility (high feasibility or low feasibility) of each indicator presented. In cases, where indicators were deemed of low relevance, indicators were not further discussed and were excluded from the list below. Shortlisted indicators presented in the tables below were voted by workshop participants to have been both highly relevant and highly feasible to collect data for (upper right quadrant seen in Figure 3.2). For some indicators workshop participants thought they were highly relevant but had low feasibility and discussion was encouraged on these indicators (upper left quadrant seen in Figure 3.2). The discussions with stakeholders aimed to understand data collection challenges and to assess whether these indicators may be shortlisted (perhaps with limitations) for the forthcoming evaluation. The independent study team ultimately made the suggestion if these indicators are included in the shortlist.

Figure 3.2 Indicators were grouped according to relevance / feasibility criteria



Source: Technopolis 2021.

3.11.1 Effectiveness

Indicators in this section consider how successful the ERNs and associated activities have been in achieving (or progressing) toward its objectives. Therefore, effectiveness indicators should measure the effects of the ERNs. Indicators for 4 dimensions of effectiveness of the ERNs were proposed: Supporting the diagnosis and treatment of patients with rare and complex diseases, Research impact on rare and low prevalence and complex diseases, Knowledge sharing helping patients with rare diseases and complex conditions to receive diagnosis and treatment and Awareness of tools available to diagnose and treat patients with rare diseases and complex conditions.

Qualitative indicators	Quantitative indicators	Data source
Supporting the diagnosis and treatment of patients with rare and complex diseases		Qualitative Interviews with HCP members of ERNs Targeted survey Case study Commission on-line public consultation Quantitative Continuous monitoring of ERNs Targeted survey CPMS
1. Perception of the CPMS system as a suitable platform to (i) exchange confidential patient data and (ii) enable collaboration across HCPs; 2. Perception of relevance of ERN registries; 3. Perception of the effects of the absence of reimbursement on the provision of panels.	1. Number of ERNs established; 2. Number of Member States with HCPs (full members and Affiliated partners) in ERNs; 3. Overall number of patients treated by members of ERNs and Affiliated Partners (if possible broken down per rare disease and complex condition); 4. Overall number of hospitals participating in ERNs (total and by MS); 5. Overall number of healthcare providers	

Qualitative indicators	Quantitative indicators		Data source
		(specialised units) participating in ERNs (total and by MS); 6. Overall number of affiliated partners (AP) represented in the ERNs; 7. Number of ERN virtual consultation panels (overall and per ERN); 8. Number of ERN registries established; 9. Change in number of rare/complex diseases covered by ERNs; 10. Number of patients in scope of all the ERNs; 11. Number of MS with legislation/process to support ERN activities.	ERN data collection Interviews with EC, ERNs and public authorities
Research impact on rare and low prevalence and complex diseases			
1. Perceived change in volume of research on rare/complex diseases in Europe; 2. Perceived change in quality of research and research collaborations on rare/complex diseases in Europe; 3. Perceived change in coverage of rare diseases targeted by research in Europe; 4. Perceived relevance of ERN registries for enhancement of research on rare/complex diseases in Europe.		5. Number of research collaborations established; 6. Number of clinical trials / studies conducted by all ERNs; 7. Number of publications by all ERNs.	
Knowledge sharing helping patients with rare diseases and complex conditions to receive diagnosis and treatment			
1. Perceived relevance and effectiveness of training content delivered by ERNs. 2. Perceived relevance of ERN Clinical Practice Guidelines. 3. Perceived change of knowledge of rare and complex diseases for HCPs within existing ERNs. 4. Perceived change of knowledge of rare and complex diseases for HCPs outside of existing ERNs. 5. Perceived change in awareness and usage of tools and resources available at the EU level for HCPs within existing ERNs; 6. Perceived change in awareness and usage of tools and resources		7. Number of training activities organised by coordination/members of all ERNs; 8. Number of healthcare providers participating in training activities; 9. Number of best practices for quality and safety benchmarks developed; 10. Number of Clinical Practice Guidelines publicly available.	

Qualitative indicators	Quantitative indicators	Data source
available at the EU level for HCPs outside of existing ERNs.		
Awareness of tools available to diagnose and treat patients with rare diseases and complex conditions		
1. Perceived change of professional awareness of the tools available at Union level; 2. Description of the effect of awareness raising activities by the EC to enhance MS cooperation;	3. Number of awareness raising activities about the Orphanet database; 4. Number of awareness raising activities about the Regulation 883/2004; 5. Share of healthcare professionals using the tools available at Union level.	

3.11.2 Efficiency

Indicators in the efficiency section consider the relationship between the level of inputs (costs and resources) available to the ERNs compared to the outputs and benefits generated to different stakeholders.

Qualitative indicators	Quantitative indicators[55]	Data source
Costs and benefits of the ERNs system		Qualitative Interviews with stakeholders Commission on-line public consultation
1. Perception of balance of costs and benefits of setting up the ERN system by stakeholder group; 2. Perception of the level of resources provided by MS to national ERN members; 3. Perception of the level of resources provided by EC to Coordinators Group, ERN coordinators and Board of Member States; 4. Perception of balance of costs and benefits of the ERN system versus traditional models of service; 5. Perception of the balance of costs and benefits of setting up the ERN system (e.g. CPMS system, website, translation tool); 6. Perception of the benefits of earlier diagnosis and access to	n/a	

[55] Please note that while costs and benefits can be quantifies and in some cases monetised, these require relevant data and suitable (health) economic models. It is therefore possible that the cost and benefits of the ERN system will rely on qualitative assessment of these aspects. As an example of the complexity of measuring benefits, see an article on quality of life benefits at: https://ec.europa.eu/eurostat/statistics-explained/index.php?title=Quality_of_life_indicators_-_measuring_quality_of_life.

Qualitative indicators	Quantitative indicators[55]	Data source
treatment in patients' quality of life; 7. Perception of the benefits of wider expertise available from experts participating in virtual consultation; 8. Perception of the costs and benefits versus traditional model.		

3.11.3Relevance

Indicators of relevance look at the changes in the needs identified at the inception of the ERNs and the current needs, and their relationship to the ERNs objectives.

Qualitative indicators	Quantitative indicators	Data source
Relevance for meeting the needs of patients with rare and complex diseases		Qualitative Interviews with patient organisations, public authorities Quantitative ERN data collection Targeted survey
1. Perceived relevance of ERNs for meeting patient needs 2. Perceived gaps in rare disease and complex conditions not covered by the ERNs	3. Number of known rare diseases and low prevalence complex conditions not covered by the ERNs	

3.11.4Coherence

Indicators in this section consider synergies of the various articles and associated actions enabled by the Directive (internal coherence) that have an impact on the implementation and governance of the ERNs. Similarly, coherence shall look at how the Directive is consistent with actions of related pieces of legislations related to the ERNs.

Qualitative indicators	Quantitative indicators	Data source
Contribution to activities on rare diseases and complex conditions such as the Orphanet database		Qualitative Interviews with EC and public authorities Case studies Quantitative ERN data collection Targeted survey
1. Perception of the complementarity and synergies created by the Directive to support the cause of rare diseases in Europe (including participation of ERNs to other EU initiatives such as EJP RD);	2. Number of MS having adopted legislation or national strategies/procedures to integrate ERNs in their national system.	

3.11.5 EU added value

Finally, indicators of added value at the EU level consider those changes that can be attributed to the EU intervention which is beyond any national action only.

Qualitative indicators	Quantitative indicators	Data source
ERN added value for patients with rare and complex diseases		Qualitative Interviews with EC and public authorities Case studies Quantitative ERN data collection Targeted survey
1. Perception of the added value the ERNs have beyond national actions by Member States;	2. Overall number of patient organisations represented in the ERNs.	

3.12 Summary of workshop discussion – Cooperation on rare diseases

In attendance at the workshop were 25 stakeholders that covered ERN coordinators and healthcare providers, one NCP, a representative from a Ministry of Health, patient representatives, as well as Commission staff as observers. The list of registered organisations is provided in 0.

The workshop discussions centred around indicators that were highly relevant but had relatively low feasibility in terms of data collection. Participants were engaged to large extent and their feedback gave insights as to which indicators are most appropriate and practical.

General points that participants raised were concerns around data collection methods for qualitative indicators. These concerns included identification of the right contacts to inform an indicator, and practical methods used to reach out to relevant contacts. One issue that was raised frequently was that the ability to collect data is variable not only from Member State to Member State, but also from ERN to ERN. One indicator may be feasible to collect for one Member State or ERN but less so for another. There was agreement among the participants that further discussion of the indicators would be of benefit. Further discussion should also include the Working Group on monitoring and other coordinators who were unable to attend the workshop.

3.12.1 Effectiveness indicators

For the **effectiveness indicators on diagnosis and treatment**, participants first raised issues with availability of data collection for the impact of an absence of reimbursement on the provision of panels. For this qualitative indicator there was suggestion that targeting ERN members to ask about the impact of an absence of reimbursement could make the indicator a feasible one to collect data for. Issues were also raised with data collection for change in time to diagnosis of patients. Despite agreement that time to diagnosis is a highly relevant indicator, feasibility of collecting quantitative such data was questioned. There was little discussion around the feasibility of data collection for indicators around the number of patients with rare disease and complex condition receiving care in another MS, however the majority voted that data

collection would not be feasible. Opinion on feasibility for the indicator number of MS with legislation/process to support ERN activities was split but there was agreement that this is an important indicator for the assessment of integration of ERNs into national systems.

The perceived change in volume of research on rare/complex diseases in Europe was the first indicator to be discussed for the section on **effectiveness indicators for research impact**. After clarification that this was intended to be a qualitative indicator there was agreement that it may be feasible to collect this indicator. Concerns were also raised around the quantitative indicator for the number of clinical trials conducted by ERNs. It was discussed that this information is more easily collected for some ERNs than for others. Clinical trials are not conducted by the ERNs themselves so data can be difficult to access. Despite the concerns, this indicator has already been included among the set of ERN key indicators by the ERN Continuous Monitoring Working Group. Further exploration into the issues that certain ERNs experience with collecting data on clinical trials, and why, is needed.

For **effectiveness indicators on awareness of tools to diagnose and treat patients with rare disease and complex conditions**, two indicators were discussed in more detail. The first was the number of awareness raising activities about the Regulation 883/2004 (quantitative). Opinion was split in the poll voting on the feasibility of collecting data for this indicator, but in the subsequent discussion clarifications were provided and suggestions were made in favour of it being accepted. There was suggestion that these activities raising awareness about the Regulation 883/2004 could come from the NCPs, and possibly collected from them. Opinion on the feasibility of data collection was similarly divided for the indicator on the number of referrals of rare disease and complex condition patients by HCPs to other Member States under the Regulation 883/2004. Issues raised during discussion of this indicator were due to differences in the processes between Member States. In some Member States this information is not systematically collected or can be difficult to obtain from the social security bodies or health insurers. For these reasons this indicator has been rejected.

Out of the **effectiveness indicators on knowledge sharing**, perceived relevance of ERN Clinical Practice Guidelines was discussed first. Although this indicator was thought of as having low feasibility during the voting poll, subsequent discussion suggested that it may be feasible due to its qualitative nature. Two other indicators were voted as being highly relevant but having low feasibility of data collection. These indicators were perceived change of knowledge of rare and complex diseases for HCPs outside of existing ERNs and perceived change in awareness and usage of tools and resources available at the EU level for HCPs outside of existing ERNs. For indicators such as these, where feasibility is low but they are highly relevant, case studies or a survey could be useful approaches for data collection. Lastly for this section, the number of each type of knowledge sharing activities across ERNs (overall and per rare disease and complex condition) was considered difficult to collect data for, as it was suggested that, at least from the NCP point of view, it is difficult to maintain an overview of ongoing knowledge sharing activities.

3.12.2Efficiency indicators

Efficiency indicator discussion centred around the difficulties associated with quantifying the costs and benefits associated with complex networks such as the ERNs, where expert advice can be provided by several experts based in many member states. Calculating costs and benefits within this complexity in a quantitative manner has many difficulties.

As such, the qualitative efficiency indicators with lower feasibility were favoured over quantitative indicators. Due to these discussions three of the quantitative indicators have been changed to qualitative indicators. These indicators were benefits of earlier diagnosis and access to treatment in patients' quality of life, benefits of wider expertise available from experts participating in virtual consultation and costs and benefits ratio versus traditional model.

3.12.3 Relevance and Coherence indicators

Only one indicator was put forward for detailed discussion out of those for relevance and coherence. All were accepted for relevance and only 'share of healthcare professionals using the Orphanet database' was suggested as having low feasibility.

3.12.4 EU added value indicators

Lastly, for EU added value, there were 2 indicators discussed in more detail. The first, perception of the added value the ERNs have beyond national actions by Member States, had low feasibility based upon the voting poll. There was no further discussion for this indicator but it appeared to be highly relevant and may be collectable due to its qualitative nature. The last indicator discussed was the number of cross-border referrals from outside the patient's country of residence (but still within EU/EEA). Discussion on this indicator confirmed difficulties with data availability in some Member States as the information is not being systematically collected by the social security bodies – as mentioned above. Due to this fact, the indicator was not considered to be of practical use.

Appendix A References

Author	Title	Description	Link
Various	Member state data on cross-border healthcare following Directive 2011/24/EU (2015-2018)	Annual MS data reports from 2015-2018	Most recent 2018 (2019 publication expected soon) https://ec.europa.eu/health/sites/health/files/cross_border_care/docs/2018_msdata_en.pdf
European Commission	Report on the operation of Directive 2011/24/EU on the application of patients' rights in cross-border healthcare (2015 and 2018)	Two reports (one in 2015 and one in 2018) on the operation of the Directive	Most recent (2018) https://eur-lex.europa.eu/legal-content/EN/TXT/?uri=COM:2018:651:FIN
European Commission	Toolbox for Cross-Border Healthcare	Intended for use by both NCPs and patients, the toolbox contains relevant information on the legal framework of cross-border healthcare	https://ec.europa.eu/health/cross_border_care/toolbox_en
Ecorys, KU Leuven and GfK Belgium	Study on cross-border health services: enhancing information provision to patients	Provides 9 guiding principles for information provision relating to the Directive whilst also including a range of indicators to measure this	https://ec.europa.eu/health/sites/health/files/cross_border_care/docs/2018_crossborder_frep_en.pdf
Footman et al	Cross-border healthcare in Europe	Policy summary of cross-border care in Europe	https://www.euro.who.int/__data/assets/pdf_file/0009/263538/Cross-border-health-care-in-Europe-Eng.pdf
ECHI	European Core Health Indicators	European Core Health Indicators database – used to explore many different health indicators and how they vary for member states	https://ec.europa.eu/health/indicators/echi/list_en
Robert Koch Institute	Data from the EU Health Monitoring Programme	Exploration of health indicators used as part of European health monitoring project	https://www.rki.de/EN/Content/Health_Monitoring/Health_Reporting/GBEDownloadsK/2012_6_european_health.pdf?__blob=publicationFile

Author	Title	Description	Link
European Commission	Guiding Principles and Indicators for the practice of National Contact Points (NCPs) under the Cross-border Healthcare Directive 2011/24/EU	The document sets out key principles for good NCP services and the voluntary list of indicators to monitor the implementation of the guidelines	https://ec.europa.eu/health/sites/default/files/cross_border_care/docs/2019_ncptoolbox_ncp_guiding_principles_crossborder_en.pdf

	ERN documentation		
European Commission	European Reference Networks: Working for patients with rare, low-prevalence and complex diseases Share.Care.Cure.	Presentation of ERNs in 2017	https://ec.europa.eu/health/sites/default/files/ern/docs/2017_brochure_en.pdf
European Commission	COMMISSION DELEGATED DECISION of 10 March 2014 setting out criteria and conditions that European Reference Networks and healthcare providers wishing to join a European Reference Network must fulfil	Document laying out the criteria that HCP need to fulfil to join an ERN	https://eur-lex.europa.eu/legal-content/EN/TXT/?uri=CELEX%3A32014D0286Commission Implementing
European Commission	COMMISSION IMPLEMENTING DECISION 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	Document laying out the evaluation process for the ERNs and their functioning	https://eur-lex.europa.eu/legal-content/EN/TXT/?uri=OJ%3AJOL_2014_147_R_0007
European Commission	COMMISSION IMPLEMENTING DECISION (EU) 2019/1269 of 26 July 2019 amending Implementing Decision 2014/287/EU setting out criteria for establishing and	Document amending previous document	https://eur-lex.europa.eu/legal-content/EN/TXT/?uri=CELEX%3A32019D1269

	ERN documentation		
	evaluating European Reference Networks and their Members and for facilitating the exchange of information and expertise on establishing and evaluating such Networks		
ERNs Board of Member States	Statement adopted by the Board of Member States on the definition and minimum recommended criteria for Associated National Centres and Coordination Hubs designated by Member States and their link to European Reference Networks	Criteria for associated centres and relations with ERNs	https://ec.europa.eu/health/sites/default/files/ern/docs/boms_affiliated_partners_en.pdf
ERN Continuous Monitoring Working Group	Continuous Monitoring of ERNs ERN Continuous Monitoring and Quality Improvement System (ERN CMQS)	List of ERN score indicators	https://ec.europa.eu/health/sites/default/files/ern/docs/continuous_monitoring_en.pdf
European Commission	4th conference on ERNs 21–22 November 2018, Brussels conference report	Conference report	4th conference on ERNs 21–22 November 2018, Brussels conference report
European Commission	European Commission. Rare Disease European Reference Networks: Addendum to EUCERD Recommendations of January 2004 (2013)	Recommendations about the grouping of RD into thematic networks and the necessity of a patient-centred approach to RD ERNs	https://ec.europa.eu/health/sites/health/files/rare_diseases/docs/20150610_erns_eucerdaddendum_en.pdf
EUROPLAN	European Project for Rare Diseases Selecting indicators to evaluate the achievements of RD initiatives	List of indicators to achieve the success of rare diseases initiatives in MS	https://download2.eurordis.org/europlan/2_EUROPLAN_Guidance_Documents_for_the_National_Conference/Proposal%20Indicators%20draft%20version%20ALIGNED.pdf

	ERN documentation		
RD-ACTION	Work package 6. Overview report on the state of the art of rare disease activities in Europe	The report highlights activities and progress at both the European Union (EU) and Member State (MS) levels	http://www.rd-action.eu/wp-content/uploads/2018/09/Final-Overview-Report-State-of-the-Art-2018-version.pdf
EUCERD	EUCERD recommendations on Rare Disease European Reference Networks (RD ERNS)	Recommendations will help focus on the criteria for the establishment and evaluation of ERNs as well as the exchange and dissemination of information.	https://ec.europa.eu/health/sites/default/files/ern/docs/eucerd_rd_ern_en_0.pdf
European Commission Rare Diseases Task Force *Working Group on health indicators*	Health indicators for rare diseases: conceptual framework and development of indicators from existing sources	Preparing the field for indicators	https://webgate.ec.europa.eu/chafea_pdb/assets/files/pdb/20082291/20082291_d04_01_oth_en_ps.pdf
European Commission Rare Diseases Task Force	European Reference Networks in the Field of Rare Diseases: State of the Art and Future Directions	Acknowledge need for indicators for ERNs	https://www.orpha.net/actor/EuropaNews/2008/doc/CE.pdf
Véronique Héon-Klin	European Reference networks for rare diseases: what is the conceptual framework?	Paper on the most important factors affecting information and knowledge exchange, as well as learning, in networks and how this can be supported	https://ojrd.biomedcentral.com/articles/10.1186/s13023-017-0676-3
Tumiene et al	European Reference Networks: challenges and opportunities	Paper introducing the challenges and opportunities of ERNs as of 2021	https://www.ncbi.nlm.nih.gov/pmc/articles/PMC7968406/
Natasha Azzopardi-Muscat, Helmut Brand	Will European Reference Networks herald a new era of care for patients with rare and complex diseases?	Paper on the potential of ERNs for patients	https://pubmed.ncbi.nlm.nih.gov/25999460/
European Commission	Rare2030 Final policy conference	Agenda of the conference	http://download2.eurordis.org/documents/pdf/Rare2030_Agenda.pdf

Source: Technopolis 2021.

Appendix B List of interviews

Organisation	Scope of activities
Acute Hospital Services, Health Service Executive Ireland	Ireland
European Commission, DG Health and Food Safety	Europe
European Commission, DG Research and Innovation	Europe
EU-PATIENTEN.DE - DVKA	Germany
EUROGEN	Europe
EURORDIS	Europe
Health Connect Partners	Europe
RIZIV-INAMI (Institut national d'assurance maladie-invalidité)	Belgium

Appendix C Participating organisations at the final stakeholder workshop

Organisation	Member State	Breakout room
Austrian Public Health Institute	Austria	Overall patient rights
Ministry of Health	Austria	Overall patient rights
Belgian Ministry of Health	Belgium	Overall patient rights
FPS Public Health	Belgium	Overall patient rights
International association of mutual benefit societies (AIM)	Belgium	Overall patient rights
National Institute for Health and Disability Insurance (INAMI-RIZIV)	Belgium	Overall patient rights
Croatian Health Insurance Fund	Croatia	Overall patient rights
Health Insurance Bureau	Czech Republic	Overall patient rights
Ministry of Health	Czech Republic	Overall patient rights
Estonian Health Insurance Fund	Estonia	Overall patient rights
Council of European Dentists	Europe	Overall patient rights
European Commission	Europe	Overall patient rights
Jonathan Olsson Consulting	Europe	Overall patient rights
Ministry of Social Affairs and Health	Finland	Overall patient rights
NCP Finland	Finland	Overall patient rights
CLEISS Paris	France	Overall patient rights
Ministry of Health	France	Overall patient rights
German Liaison Agency Health Insurance - International (DVKA)	Germany	Overall patient rights
Federal Ministry of Health	Germany	Overall patient rights
NCP Germany	Germany	Overall patient rights
EOPYY National Organization for the Provision of Health Services	Greece	Overall patient rights
Ministry of Human Capacities	Hungary	Overall patient rights
Icelandic Health Insurance	Iceland	Overall patient rights
Department of Health	Ireland	Overall patient rights
Health Service Executive	Ireland	Overall patient rights
National Health Service	Latvia	Overall patient rights
National Health Insurance Fund	Lithuania	Overall patient rights
State Health Care Accreditation Agency	Lithuania	Overall patient rights
State Patient Fund	Lithuania	Overall patient rights
Ministry For Health	Malta	Overall patient rights
euPrevent	Netherlands	Overall patient rights
Ministry of Health, Welfare and Sport of the Netherlands - Health Insurance Department	Netherlands	Overall patient rights
NCP Netherlands (CPK)	Netherlands	Overall patient rights
National Health Foundation	Poland	Overall patient rights

Organisation	Member State	Breakout room
National Health Insurance House	Romania	Overall patient rights
Ministry of Health	Slovakia	Overall patient rights
Ministry of Health	Spain	Overall patient rights
National Board of Health and Welfare	Sweden	Overall patient rights

Organisation	Member State	Breakout room
St. Anna Children's Cancer Research Institute (CCRI)	Austria	Cooperation concerning rare/complex diseases
Rare Diseases Europe (EURORDIS)	Belgium	Cooperation concerning rare/complex diseases
MetabERN	Belgium	Cooperation concerning rare/complex diseases
AVMinority	Czech Republic	Cooperation concerning rare/complex diseases
The Czech Association for Rare Diseases	Czech Republic	Cooperation concerning rare/complex diseases
European Commission	Europe	Cooperation concerning rare/complex diseases
EUREGHA	Europe	Cooperation concerning rare/complex diseases
European Social Observatory (OSE)	Europe	Cooperation concerning rare/complex diseases
Standing Committee of European Doctors (CPME)	Europe	Cooperation concerning rare/complex diseases
Assistance Publique - Hôpitaux de Paris (ERN ITHACA)	France	Cooperation concerning rare/complex diseases
ERN EPICARE	France	Cooperation concerning rare/complex diseases
ERN Euro-NMD	France	Cooperation concerning rare/complex diseases
ERN EuroBloodNet	France	Cooperation concerning rare/complex diseases
ERN LUNG	France	Cooperation concerning rare/complex diseases
Hospices Civils de Lyon	France	Cooperation concerning rare/complex diseases
University Hospitals of Strasbourg	France	Cooperation concerning rare/complex diseases
ERN LUNG (Universitätsklinikum Frankfurt)	Germany	Cooperation concerning rare/complex diseases
University Medical Center Hamburg-Eppendorf (UKE)	Germany	Cooperation concerning rare/complex diseases
ERN ReCONNET	Italy	Cooperation concerning rare/complex diseases
MetabERN	Italy	Cooperation concerning rare/complex diseases
Children's Clinical University Hospital	Latvia	Cooperation concerning rare/complex diseases
Radboudumc	Netherlands	Cooperation concerning rare/complex diseases

Organisation	Member State	Breakout room
University Medical Center Utrecht	Netherlands	Cooperation concerning rare/complex diseases
Pomeranian Medical University in Szczecin	Poland	Cooperation concerning rare/complex diseases
ERN TransplantChild	Spain	Cooperation concerning rare/complex diseases
ERN GENTURIS (Hospital Germans Trias)	Spain	Cooperation concerning rare/complex diseases

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