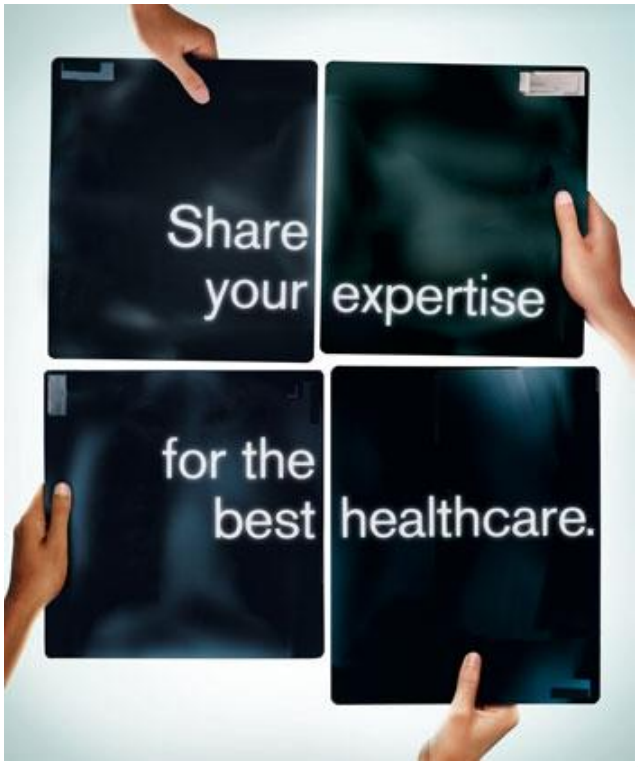
European Reference Networks

Share. *Care*. Cure.

Literature Review on practices and methods on the assessment of highly specialised healthcare providers in the European Union



An initiative of the European Commission

September 2015

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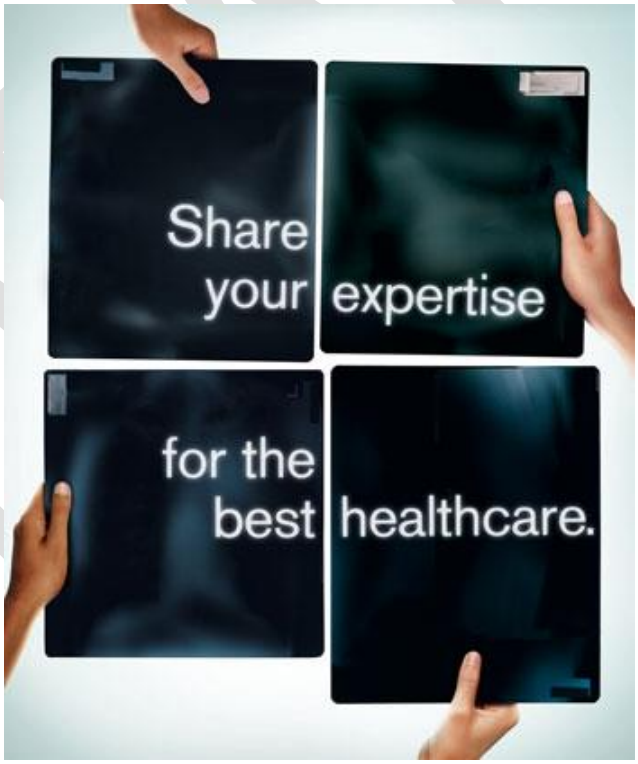
Literature Review part I

on Assessment Methods for Healthcare Services



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Literature review part I: on Assessment Methods for Healthcare Services

1. Executive Summary

1.1 Introduction

This literature review has been conducted by the Partnership for Assessment of Clinical Excellence in European Reference Network (PACE-ERN) consortium, under the framework of a contract with the European Commission to develop a technical proposal for an Assessment Manual and Toolbox (AMT) for European Reference Networks (ERNs).

1.2 Objectives

The purpose of the literature review for the ERN project is to identify published evidence related to assessment, particularly where it relate to networks and services for rare, low prevalence or complex diseases.

1.3 Methods

This literature review is based on standard methods. English language articles, published between 2000 and 2015, where retrieved from PubMed using an inclusive keyword based search strategy. These were then screened for relevance before being sorted, evaluated and the themes from them drawn together.

The methods used for assessment included: use of data (routine and service specific), service based evaluations (visits and interviews with staff and patients), questionnaires (surveys, staff and patient questionnaires), and case reviews (medical record reviews and second opinions).
As well as identifying the methods of assessment currently in use, a strong theme was found regarding the necessity and appropriate use of the patient‘s perspective in assessment.

2. Introduction

2.1 Context of the Literature Review

The European Commission (EC) contracted the Partnership for Assessment of Clinical Excellence in European Reference Network (PACE-ERN) consortium to develop a technical proposal for an Assessment Manual and Toolbox (AMT) for European Reference Networks (ERNs) based on the Delegated and Implementing Decisions for establishing and evaluating ERNs (March 10, 2014).

The purpose of the literature review for the ERN project is to identify published evidence related to assessment, especially relating to networks and services for rare, low prevalence or complex diseases. The questions that are explored in this literature review:

- What are the methods used to evaluate networks and services for rare, low prevalence or complex diseases?
- What are the methods used to evaluate networks or services in some of the common diseases and injuries (e.g. trauma)?
- What are there transferrable elements that could help in the assessment of ERNs?
- What other information from scientific publications can be used to inform the development of the ERN assessment programme? For example: what aspects of network/service delivery are being assessed?

2.2 Assessment in perspective

Accreditation is broadly viewed as being beneficial to all stakeholders and that the benefits generally outweigh the burden it imposes (Valori et al., 2013). Accreditation has been suggested as having a strong association with patient outcomes across a range of contexts and areas (for example Organisation wide (Braithwaite et al., 2010, Greenfield et al., 2014), Stroke Units (Stroke Unit Trialists' Collaboration, 2007), Endoscopy (Bhangu et al., 2012, Stebbing, 2011), Mental Health Services (Murphy et al., 2013) and Laboratory Services (Tan et al., 2004)). Accreditation has, for example, been shown to reduce variation and maximise the quality of colonoscopic performance in the UK (Bhangu et al., 2012). Giangrande et al (2014) have similarly suggested that the implementation of European Guidelines for the certification of Haemophilia Centres contribute to the reduction of health inequalities through the standardisation of care. For many health services, the accreditation process is viewed as a motivation to achieve best practice, a positive event from which credibility for current practice and future direction can be gained (Greenfield et al., 2014).

Braithwaite et al (2010) reported that service accreditation was a predictor of clinical and organisational performance in their study of acute care in Australia. They found that accreditation performance was an accurate reflection of factors that are important in influencing the quality of care and continuous improvement. Similarly Beaulieu and Scutchfield (2002) found that whilst National Public Health Performance Standards in the US raised concerned about their achievability, they were nonetheless valid measures of performance.

The effective delivery of accreditation systems is complex, however. They are most effective when medical institutions are an active agent in the accreditation rather than a passive recipient of compulsory external evaluation (Chang and Lee, 2012). Such accreditation is best seen as a process of continued business improvement rather than a snapshot of activity, and therefore effective accreditation requires continuous communication from all parties: medical institution, government and accreditation agency. All accreditation systems have their strengths and limitations, which places particular demands on the sensitive and appropriate ways in which they are developed and applied (Beutler, 2001). It cannot be assumed that quality improvement tools in one setting are necessary applicable to others: the rationale and appropriateness of their use has to be considered carefully (Crossland et al., 2014). Without a clear understanding of the outcomes of accreditations, there will inevitably be confusion over the endpoints of the process, 'especially for government mandated programmes where accreditation is often used more for regulation and public accountability rather than as a means of voluntary self-assessment and quality improvement' (Mumford et al., 2013:608).

3. Methods

3.1 Model

This literature review is based on a standard methodological framework. This literature review involved a rapid review approach, informed by adapted systematic review design:

Search terms
Search engine (inclusion and exclusion)
Abstract screening
Retrieving papers
Extracting data using criteria informed by study aims
Narrative synthesis

3.2 Search Method

To complete the literature review, the national library of medicine Medline electronic database was consulted with the search updated and correct on 14th May 2015.

Inclusion criteria

English language
Date range 2000 - 2015
No limitations were placed on study designs.

(For the formal search terms see Appendix I)

The search yielded 1782 papers.

3.3 Screening the Results

Initial screening of the title for papers potentially relating to the assessment of networks or services reduced the number to 253.

Abstract reviewing and screening resulted in 66 papers that address aspects of method for assessing services or networks and a further 75 papers of wider relevance that were felt might be useful to inform the development of the ERN assessment programme.

3.4 Sorting and Collating the Results

Four broad categories emerged iteratively during the reading of papers and were subsequently used to structure the review findings:

Methods for conducting assessments - 66 papers
General evidence regarding assessment - 42 papers
The patient perspective - 16 papers
Elements to assess - 17 papers

For the purposes of collating the data, each paper was allocated to only one of the broad categories; the one it made the most significant contribution to. This process was done only for the purpose of classification and organisation of the data. Texts were allowed to contribute to whichever section or sections they contained useful information for.

Methodological papers were categorised further into the specific methods used, although mixed method approaches were the most common. The individual methods included:

Data (Routine health system, Service specific)
Service Based (Visit, Staff Interviews, Patient Interviews)
Questionnaires (Service Survey, Staff questionnaires, Patient Questionnaires)
Case Review (Medical Record review, 2nd Opinion)

3.5 Approach to Summarising and Synthesising the Evidence

The approach to summarizing and synthesizing the evidence was developed to ensure that this literature review presented the optimum evidence to inform the development of the ERN assessment programme. It primarily focuses on drawing together information on the models and methods for assessment.

There were few examples of where papers presented a rigorous evaluation of methods used to assess health care centres, networks or processes. There were few examples of methods specific to rare, low prevalence or complex diseases. It was typical for papers to describe the methods of assessment used and then present the findings of the evaluation with a brief reflection on the limitations of the approach. It became evident after a cursory overview of the evidence that at best this literature review would only

be able to describe the methods used, in various contexts and highlight any perceived benefits, drawbacks, comments or issues with those methods.

The approach taken was therefore directed by the nature of the evidence and a realist type approach was used (what works, for whom, and under what circumstances) (Wong et al., 2013, Wong et al., 2014).

4. Results

4.1 General Evidence Regarding Assessment

4.1.1 The Act of Measuring

The act of measuring alone is often cited as a significant stimulus to quality improvement in healthcare. Successful assessment and accreditation programmes often include a phase of supported self-assessment against the standards (Charles et al., 2011, Foster et al., 2007, Kirschenbaum et al., 2010, Valori et al., 2013, Winship and Lee, 2012) irrespective of the other methods that the assessment programme uses. This has a particular benefit of developing an orientation towards quality rather than simply as a regulatory barrier that must be past.

4.1.2 Mixed Methods

Each method of assessment has strengths and weaknesses (see Table 1 - Summary of Assessment Methods), and because of this, it is common for healthcare assessments to include multiple methods (For example: Ovretveit and Klazinga (2012), Tafese et al. (2013), and Tesfaye and Oljira (2013)). Tumlinson et al (2014:462) have demonstrated that whilst certain methods accurately measured quality indicators for family planning services, each had its own drawback: there was therefore a 'need for conceptual clarity in defining, measuring, and analysing quality of care' when selecting the methods to be used to assess services.

The approach of using multiple methods allows each method to be used where it is strong and for triangluation of information (Carter et al., 2014, Patton, 1999). Tafese et al (2013), for example, triangulated their data on client interviews with users of family planning services in Ethiopia by drawing on information from provider interviews and inventory checklists. One technique that can be used to draw multiple methods together effectively is tracer methodology (Chang and Lee, 2012). This approach follows patients, specific high-risk patient groups or system processes through use of data, case records and service evaluation visits.

A mixed method approach can often provide a more robust understanding of issues or assessment of quality. For example, qualitative approaches (such as semi-structured interviews or focus groups) can be used to provide an explanation or context to quantitative data and conversely quantitative approaches (such as using national data sets or survey design) can be used to map the extent of issues identified in qualitative data.

Examples of mixed methods include Agha et al (2009), who combined service-based questionnaires with health provider interviews, observations and exit interviews with clients in their evaluation of family planning services in Kenya. Similarly Tesfaye and Oljira (2013) used patient interviews, direct service observation, provider self-administered questionnaires and an inventory of equipment and supplies in their evaluation of post abortion care in Ethiopia.

4.2 Methods for Conducting Assessments

In a cross-sectional study Braithwaite et al. (2012) noted that while there was a consistent model of accreditation worldwide, centring on promoting improvements, applying standards and providing feedback. There is a divergence with regard to specialised features between Low and Middle Income Countries (LMICs) even if the general logic is the same. LMICs were less likely than HICs to include an evaluation component to programmes, and were more likely to make decisions on the accreditation status based on a formulaic, mathematically oriented approach.

Previously conducted literature reviews on assessment methods have shown that there is a wide range of approaches adopted. For example, evaluations of private pharmacy services in LMICs have used many different methods ranging from staff questionnaires to assessments of practice using simulated client methodology (Smith, 2009). As with all methods, there is no single tool or approach that is better than others in evaluating health services: they all have inherent strengths and weaknesses. What is important is that the context of the evaluation in terms of desired evaluation outcomes, the characteristics of the service design (including staff) and the characteristics of the population groups are considered when selecting evaluation methods.

This section sets out the core evaluation methods used with illustrative examples and describes their use in a variety of contexts, both geographical and by service type.

4.2.1 Data

One method of evaluating services is the reuse of data that already been collected under different contexts. Such data sets typically occur either within the particular health care hospital / centre / unit or at a national / international scale. National data sets have been used to assess inpatient palliative care services across Korea (Choi et al., 2012), hospital payment schemes in Italy (Cavalieri et al., 2013), cancer network management in England (McCarthy et al., 2008) and trauma centres in the US (Plurad et al., 2011). These studies typically use national data sets to map variations in service provision. Plurad et al (2011), for example, used the National Trauma Databank to assess trauma centre designation and the incidence of post-traumatic acute respiratory distress syndrome, reporting a variation in management between centres providing similar services. Similarly, data from the Korean Terminal Cancer Patient Information System, which consisted of patient- and episode-level data, was used to highlight variations on mean length of stay, subsequent place of care and change in average pain score across inpatient palliative care services in Korea (Choi et al., 2012).

It is seen to be both ethical and efficient to reuse national data sets for purposes outside their original design. National data provided by the NHS, government agencies and the Department of Health in the

UK, for example, provide important information of population incidence and survival. However, they have also been used at the hospital level for management purposes, comparing cancer networks in England on indicators such as waiting times (McCarthy et al., 2008). Similarly, it has been noted that the use of Hospital Episode Statistics as grown considerably in the UK, although the use of such data is not unproblematic, with problems relating to generic and context-specific reporting being identified (Sinha et al., 2013).

National data can be used in more sophisticated ways than mapping regional variations in practice (Coles et al., 2012). They can also be used to examine whether particular characteristics of health providers are a predictor for better health outcomes. For example, data from the National programme for Outcome Assessment in Italy were used to assess mortality and readmissions for Acute Myocardial Infarction, Congestive Heart Failure, stroke and Chronic Obstructive Pulmonary Disease. The study showed that hospitals operating in regions where prospective payments are used more extensively are associated with better quality of care as defined by mortality and readmission metrics (Cavalieri et al., 2013).

Routine data collected within specific health services has also been reused for quality assessment purposes. Examples include the use of discharge data to examine the impact of an experienced acute care nurse practitioner on trauma services in the US (Collins et al., 2014), significant event analysis reports to assess two General Practitioner groups in Scotland (Bowie et al., 2008), hospital data (e.g. preoperative length of stay) to assess the impact of an Acute Surgical Unit in New Zealand (Hsee et al., 2012), and the use of registry information (e.g. disease duration) to assess the quality of care for inflammatory bowel disease in Sweden (Rejler et al., 2012). The evaluation of the impact of acute care nurse practitioners analysed discharge data collected on the institution's software, which is used for admission, discharges, transfers in the hospital, chart abstraction, billing and length of stay (Collins et al., 2014). The evaluation reported that the addition of experienced acute care nurse practitioners result in the decrease of overall trauma service length of stay, saving almost $9m in hospital charges.

Data systems have been established within health services for the specific purpose of service evaluation. Clinical quality in rare disease services in England, for example, have been evaluated using case series outcomes data on death, survival, mortality and median survival time collected by the participating hospitals themselves (Kenny et al., 2008). The quality of care and financial impact of a virtual renal clinic was assessed by recording data on a specifically designed renal database, including patient demographics, referral indication, clinical history, medical comorbidities, previous investigations, management advice provided and mode of follow up (Mark et al., 2011).

Various strategies have been used to determine which key performance indicators and evaluation criteria should be applied in health care evaluations. Kenny et al (2008) reflected that it was helpful if outcomes are agreed with the clinicians in advance rather than being imposed in an effort to maintain constructive relationships between commissioners and services and to maximize compliance.

4.2.2 Service Based Evaluations

Service based evaluations employ a range of methods, typically qualitative in design. Methods include visits by peers or evaluation teams, participatory observations, qualitative interviews with patients or staff and focus groups. Visits by external teams have been used in evaluations of the National Cervical Cancer Screening Programmes of Bangladesh (Basu et al., 2010), and the review of quality of care by the American College of Obstetricians and Gynaecologists (Stumpf, 2007). Øvretveit and Klazinga (2012) describe the generation of evaluation hypotheses used by evaluation teams to assess the implementation success of national health and social care quality programmes in the Netherlands, including a literature review to identify factors critical to the success of large-scale improvement programmes. The evaluation of Bangladeshi National Cervical Cancer Screening Programmes employed external reviewers comprising a reviewer with experience in field research on relevant screening methods and a consultant experienced in performing quality assurance evaluation (Basu et al., 2010). They found data collection was not systematic and quality standards and monitoring / quality assurance plans were poorly defined. The external reviewers generated a protocol for continued internal quality assurance (Basu et al., 2010).

Numerous studies have employed observation methods in their evaluation design (Agha and Do, 2009, Charles et al., 2011, Ith et al., 2012, Kinkel et al., 2012, Tafese et al., 2013). Ith et al (2012), for example, used structured non-participant observation methodology in their assessment of the quality of maternity care practices of skilled birth attendants in Cambodia. Data were collected using an observation checklist based on the national clinical assessment tools for the associate degree in midwifery developed by the Cambodian Ministry of Health and United Nations Population Fund. The evaluation reported that the childbirth practices of skilled birth attendants were not consistent with evidence-based practice (Ith et al., 2012). Another model of observations was used by Kinkel et al (2012) in their assessment of antiretroviral treatment clinics in South Africa. Trained participant observers presented as patients and evaluated each facility on five difference occasions, assessing the time it took to get an appointment, the services available and accessed, service quality and the duration of the visit. The assessment identified variations in service quality and specific problem areas that needed to be addressed.

Qualitative interviews have similarly been used, typically being conducted on staff members (Kampirapap et al., 2005, Kavalieratos et al., 2014, Mannion and Goddard, 2001) and patients / clients (Kampirapap et al., 2005, Karkee et al., 2014, Tesfaye and Oljira, 2013). Kampirapap et al (2005) conducted semi-structured interviews with patients, community members and health staff in their assessment of the quality of leprosy services in Thailand. The use of qualitative interviews enabled the staff to generate much rich data on patient views and allowed new and interesting information to be generated. It also allowed a deeper understanding of the context of problem areas and patients behaviours / activities to be understood (Kampirapap et al., 2005). Semi-structured interviews were also conducted with physicians, nurse practitioners and physician assistant providers in an assessment of factors impeding palliative care referral for heart failure patients, and again were helpful in exploring the context and nature of perceived barriers (Kavalieratos et al., 2014).

Alternatively, Mannion and Goddard (2001) conducted semi-structured interviews specifically with chief executives, medical directors and other key staff in their evaluation of the impact of the publication of clinical outcomes data on NHS Trusts in Scotland. The authors noted that in the evaluation, the use of indicators helped to raise the awareness of quality issues and alerted providers to specific areas.

In one study, focus groups were used to explore patient, community member and health staff views identified in earlier semi-structured interviews in greater depth (Kampirapap et al., 2005). Focus groups are often seen as useful adjunct to qualitative interviews, for, whereas interviews identify thoughts, feelings and experiences in a one-to-one setting, a group setting can be viewed as being more 'realistic'.

In addition to the benefit to a service of engaging with a process of accreditation, there are benefits that are not obvious to initial examination. For example peer-review assessors find the process of visiting or reviewing medical records supportive of their own personal and professional development and they invariably take back to their own organisations new ideas and ways of working (Valori et al., 2013).

4.2.3 Questionnaires

Questionnaires are a common evaluation tool, being distributed at a service level (e.g. (Gavin et al., 2013, Perera et al., 2007, Srofenyoh et al., 2012)), to health care staff (e.g. (John et al., 2010, Lane and Bragg, 2007, Tafese et al., 2013)) and most typically to patients (e.g. (de Oliveira et al., 2006, Harnett et al., 2010, Lemos et al., 2014, Nekoei-Moghadam and Amiresmaili, 2011, Rejler et al., 2012, Tourigny et al., 2010)). Service level surveys are typically audits (Gavin et al., 2013), but can include the application of appraisal tools (Perera et al., 2007). For example, a national colonoscopy audit was conducted involving 302 units in participating UK NHS hospital (Gavin et al., 2013). The audit was collated via a website with the nursing team responsible for the collection of key performance indicators, which were then compared to a previous survey. The audit demonstrated an improvement in the quality of colonoscopy in the UK with performance being above the required national standards. Nationally driven interventions of training and quality improvement were seen as key reasons for this improvement (Gavin et al., 2013).

An indicator appraisal tool was used to assess performance in New Zealand primary care services (Perera et al., 2007). The appraisal tool combined the assessment of scientific evidence with contextual considerations from the perspective of the policy environment and the primary health care sectors. The theoretical framework was designed on the basis of a literature review and interviews with key informants.

Staff surveys have been used in a variety of contexts. Service provider self-administered surveys have been used to assess the quality of post abortion care in health facilities of the Guraghe Zone of Ethiopia (Tesfaye and Oljira, 2013). The evaluation reported satisfactory interactions between patients and service providers, but noted that specific areas of clinical service delivery were being neglected. By contrast, John et al (2010) used postal questionnaires to all rheumatology consultants, and clinicians in the West Midlands, UK to rate audits coordinated by the West Midlands Rheumatology Service and Training Committee. The survey found that there was general agreement that audits benefited patients,

provided the potential for improving hospital services and allowed different units to be compared (John et al., 2010).

Patient / client surveys have been used to evaluate the quality of maternity services in Nepal (Karkee et al., 2014), health-service treatment for TB in Brazil (Lemos et al., 2014), and the quality of family planning services in the Jimma Zone, Ethiopia (Tafese et al., 2013). Several studies used the survey approach to recorded patient satisfaction of services. Harnett et al (2010), for example, used two yearly cycles of patient satisfaction surveys to assess the process and impact of changes within a preoperative clinic in a US Tertiary Teaching Hospital. Survey questions were related to explanations of clinic by the surgeon's office, courtesy and efficiency of the clinic staff, and waiting time. The study reported that scores were higher for all questions in the second round, indicating improvement in practice (Harnett et al., 2010). Similarly before and after comparisons have been made, using patient surveys of primary care reform in Canada (Tourigny et al., 2010). In this study, five family medicine groups were assessed at the beginning of their implementation and then eighteen months later. The survey revealed that certain aspects of care improved after the implementation of family medicine groups, such as willingness to see nurses, whilst others did not improve, such as accessibility (Tourigny et al., 2010).

4.2.4 Case Review

Care reviews were either medical record reviews (Kirschenbaum et al., 2010, Kroger et al., 2007, O'Hara et al., 2012) or second opinions of samples (Ellis et al., 2006, Harnden et al., 2008, Schneider et al., 2005). O'Hara et al (2012), for example, conducted a retrospective patients care note review to compare the quality and safety of care provided by emergency care practitioner and non-emergency care practitioners (e.g. nurse practitioners) across three different types of emergency care settings: static services, ambulance/care home services, and primary out of hours services. The evaluation showed that the overall care of an extended skill role, such as emergency care practitioners, scored more highly than non-emergency care practitioners. However, the authors noted that a limitation of the record review methodology is that reviewers were reliant on information recorded in case notes, which may be at a level of detail that reflects all the aspects of the care delivered (O'Hara et al., 2012).

Several studies used second opinions. Ellis et al (2006) examined the impact of a national external quality assessment scheme for breast pathology by preparing three sets of stained histological sections from single blocks of 12 cases to 17 regional coordinators in the UK for circulation to pathologists in their regions. They reported that consistency was high amongst pathologists and they were able to track significant and sustained improvement in grading after the release of revised guidelines in 1995 (Ellis et al., 2006). Schneider et al (2005) assessed the impact of diabetic retinopathy telescreening on patients recruited from screening sites in five European countries. Patients attending each clinic's diabetic retinopathy screen service received standardised retinal photography. The images and related data were then transferred electronically to a remote location for grading. A systematic quality management approach was used to assess each photographer uploading images and each grader downloading images. Assurance measures used included image quality and intragrader reliability. The evaluation indicated that the telemedicine-supported quality assurance process was both practical and advantageous (Schneider et al., 2005).

Table 1 - Summary of Assessment Methods

		Location	Effort/ intensity	Ability to assess		
				Structure	Process	Outcome
Data						
	Routine health system	Remote	+	+	+	+
	Service specific	Remote	++	++	++	++
Service Based						
	Visit	Local	++	+++	+++	+
	Staff Interviews	Local	+++	+	+++	++
	Patient Interviews	Local	+++	+	+++	++
Questionnaires						
	Service Survey	Remote	+	++	++	+
	Staff	Remote	+	++	++	+
	Patient	Remote	+	+	+++	++
Case Review						
	Medical Record Review	Local	+++	+	+++	+++
	2nd Opinion	Remote	++	+	+	+++

4.3 The Patient Perspective

The perspective of patients is a recurrent theme from much of the literature on assessment. It is a critical juncture covering both how to assess and what to assess. Patients can bring valuable views to Visits (Kinkel et al., 2012), Designing Questionnaires (Stevenson et al., 2004, Blais et al., 2002, Vargo et al., 2013) and ensuring that processes bring best value to assessment (Perreault et al., 2010, Syed et al., 2015).

Patient perspectives are complex (Fotaki et al., 2008) and they often emphasise personal relations and co-operation with the staff, considering them more important than technical factors (Jakobsson and Holmberg, 2012). However the emphasis patients put on issues vary and there is a need to elicit information from patients on what matters to them for each particular issue rather than assuming what matters. It is important to ensure that assessment processes are patient centred that patients' views on service quality are sought, and that patients are engaged with the design of the assessment process itself.

Patient population groups are not an undifferentiated whole. Different characteristics have been shown to affect rating of quality, satisfaction and needs assessment. For example, the exercise of choice and information use is higher for better-educated populations (Fotaki et al., 2008). Similarly, education level and gender has been shown to influence ratings of services, where women typically rate services lower in quality (Pini et al., 2014).

The single most important element to assess with regard to patients would appear to be their experience of service quality or satisfaction. Patient experience of service quality is important enough that it is a predicator of survival in some cancers (Gupta et al., 2013, Gupta et al., 2014). When the collection and analysis of routine data is well designed it can be used to help interpret information on patient satisfaction gathered via other methods (McCarthy et al., 2009) underlining the argument for mixed methods.

Clinical services often overestimate the service they provide when compared to surveys of their patients (Stevenson et al., 2004). Those services that provide better customer services and access also provide better clinical care (Schneider et al., 2001).

Evaluators have noted the advantages that patient and public involvement bring to evaluation design. In their work on evaluating inpatient psychiatric care, Blais et al (2002) discussed the help patients provided in designing the evaluation tool and were thus seen to be instrumental in generating meaningful results that aided the survey design. Participatory designs have similarly been shown to be effective in the design and delivery of quality assessment activity (Vargo et al., 2013). They have been used to develop a framework for assessing quality of care in children's mental health services in the US where participants were actively engaged in identifying health needs, interventions and assessments of their effectiveness (Vargo et al., 2013).

4.4 Elements to Assess

What specific elements to assess appears to be less important than the process by which those elements are selected. Commonly Delphi processes are used to get to an agreed and supported list of indicators (Engels et al., 2005, Ivorra et al., 2013, Kroger et al., 2007, Woitha et al., 2014, Zhao et al., 2015). Especially in services covering rare, low prevalence or complex conditions where there is low expectation that specific data of interest for the assessment of these services will be well covered in system wide routine data. There are examples of processes of agreement between the assessing body and prospective services (Kenny et al., 2008).

The next section will highlight the kind of information that was found within this review of the literature to have been effectively collected from the methods described above.

4.4.1 Data

There are innumerable approaches regarding which data to collect and use for assessment. The approaches range from the selection of a limited number of condition specific indicators collected at the level of the service (Kim et al., 2012b, Kenny et al., 2008) through to routine national datasets (Kyei et al., 2012, Sinha et al., 2013).

Routine data can evaluate outcomes where there are objectively measured parameters that are effective surrogates (e.g. HbA1c, cholesterol or creatinine) (Whitford et al., 2004) or final end-points e.g. mortality (Nobilio et al., 2004, Kim et al., 2012a, Chinachoti et al., 2002, Selim et al., 2006, Srofenyoh et al., 2012).

National data sets have been interrogated to assess a wide variety of metrics from population incidence, survival, recurrence, mortality, through to Hospital Episodes, waiting times and readmissions (Sinha et al., 2013, Cavalieri et al., 2013, Coles et al., 2012, Kim et al., 2012a, McCarthy et al., 2008). As we saw in Section 4.2, such data can be used to map variation in practice or performance, or changes over time, particularly in relation to the implementation of changed practice. Similarly, a wide range of data within hospitals has been collected to compare performance between centres or against national guidelines, or to track change over time. Such data may include information on discharge, length of stay, disease duration or adverse incidents (Bowie et al., 2008, Collins et al., 2014, Hsee et al., 2012, Rejler et al., 2012).

4.4.2 Service Based Evaluations

Peer reviews of services are able to appraise the infrastructure within a service (McCarthy et al., 2008) and they are often able to assess consistency of practice with guidelines and the quality of the service management (Heaton, 2000, McCarthy et al., 2008, Stumpf, 2007).

Interviews with staff members can give detailed information about attitudes to patients and about the clinical processes and procedures followed in the service (Kampirapap et al., 2005, Kavalieratos et al., 2014, Mannion and Goddard, 2001). Interviews with patients can give accurate insight regarding the experience of care within the service and about things that staff assume should be in place but are not necessarily there (Kampirapap et al., 2005, Karkee et al., 2014, Tesfaye and Oljira, 2013).

Evaluation visits and observations typically use observation checklists to assess practices. Such checklists are often informed by Quality Assessment agency criteria, clinical guidelines or a review of the literature and have included the time it took to get an appointment, the services accessed, duration of the visit, communications and general interactions between patient and provider (Agha and Do, 2009, Charles et al., 2011, Ith et al., 2012, Kinkel et al., 2012, Tafese et al., 2013).

Qualitative interviews by contrast typically explore attitudes and behaviours, uncovering the context and reasons why problems occur or conversely where and why interventions are successful (Kampirapap et al., 2005, Karkee et al., 2014, Kavalieratos et al., 2014, Mannion and Goddard, 2001, Tesfaye and Oljira, 2013).

4.4.3 Questionnaires

Questionnaires in the form of service surveys are able to identify differences between practice in services, but they are not often able to evaluate the service (Cherlin et al., 2011). Surveys are particularly suited to collecting information on infrastructure, staffing, information systems, finance and quality or safety procedures (Engels et al., 2005).

Well-designed questionnaires to patients and/or staff members can provide effective tools for qualitatively tracking aspects of the services. Although without additional triangulated information from other methods, the results of questionnaires can be difficult to interpret.

Service level questionnaires are often audits or facility inventories, collecting unit wide data on activities and key performance indicators (Gavin et al., 2013, Perera et al., 2007). Surveys to staff can similarly be used to record activity but have also been used to rate attitudes towards quality improvement activities or document evidence of impact and change (John et al., 2010, Tafese et al., 2013). As discussed in section 4.2, patient surveys often include information on satisfaction on aspects of care or to identify need and indicate the relative importance of services provided (Tafese et al., 2013, Tourigny et al., 2010, Harnett et al., 2010, Lemos et al., 2014).

4.4.4 Case Review

Reviews of medical case records can provide valuable insight into the processes of care and the outcomes achieved (Irons et al., 2008, Nasic et al., 2005, O'Hara et al., 2012, Suriyawongpaisal et al., 2012).

Assessments of laboratory services often include some element of quality assurance through blinded peer-review or second opinion regarding cases (Ellis et al., 2006, Goldie, 2001, Harnden et al., 2008, Maramba, 2002, Tan et al., 2004, Valenstein et al., 2009). These methods are relatively easier when the case under scrutiny is a sample of some kind. There are no examples in the published literature of equivalent methods where patients are seen by multiple services for the purposes of assessment of the quality of a service.

4.4.5 Other Elements Identified in the Review

The quality of leadership has a positive impact on quality, mediated through quality improvement programmes. An important marker of quality of leadership is the presence of directors with masters degrees (Carman et al., 2014).

When services are involved in Quality Improvement activity this can be a specific process marker to be looked for during assessment because it is often associated with improving outcomes (Carman et al., 2014, Greenfield et al., 2014, Hodges and Wotring, 2004, Ovretveit and Klazinga, 2012, Bick et al., 2011).

Increasing specialist volume is a factor that is not always clearly associated with improved outcomes. However it is often easily measured and, as well as there being evidence for minimum levels to maintain competence, there are many examples of the positive association between volume and outcome (Bhangu et al., 2012, Bulliard et al., 2011, Hsee et al., 2012, Kim et al., 2012a, Lau et al., 2014, Nobilio et al., 2004). There is also an association between experience, characterised by ‘hands-on, time served’ (Bradt and Drummond, 2007).

5. Appendix 1 - Search Terms

Search, Query, Items found,

#17,"Search (#14) NOT #20",1782,
#16,"Search (#14) NOT #15 Filters: Publication date from 2000/01/01 to 2015/05/01; Humans; English",1782,
#15,"Search quality of life",244212,
#14,"Search (#9) AND #12",2344,
#13,"Search (#9) AND #12 Filters: Publication date from 2000/01/01 to 2015/05/01; Humans; English",2344,
#12,"Search (((expertise) OR quality) OR specialist) OR specialized",920893,
#11,"Search (quality) AND #9",1916,
#10,"Search (quality) AND #9 Filters: Publication date from 2000/01/01 to 2015/05/01; Humans; English",1916,
#9,"Search (#6) NOT #7",6831,
#8,"Search (#6) NOT #7 Filters: Publication date from 2000/01/01 to 2015/05/01; Humans; English",6831,
#7,"Search health need",207722,
#6,"Search (#3 AND #2) Filters: Publication date from 2000/01/01 to 2015/05/01; Humans; English", 8216,
#5,"Search (#3 AND #2) Filters: Publication date from 2000/01/01 to 2015/05/01; Humans",8960,
#4,"Search (#3 AND #2) Filters: Humans",11930,
#3,"Search service[Title/Abstract] Filters: Humans",126590,
#2,"Search ((((assessment[Title/Abstract]) OR accreditation[Title/Abstract]) OR certification[Title/Abstract]) OR designation[Title/Abstract]) OR licensing[Title/Abstract] Filters: Humans",493185,
#1,"Search ((((assessment[Title/Abstract]) OR accreditation[Title/Abstract]) OR certification[Title/Abstract]) OR designation[Title/Abstract]) OR licensing[Title/Abstract]",657441,

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European Reference Networks

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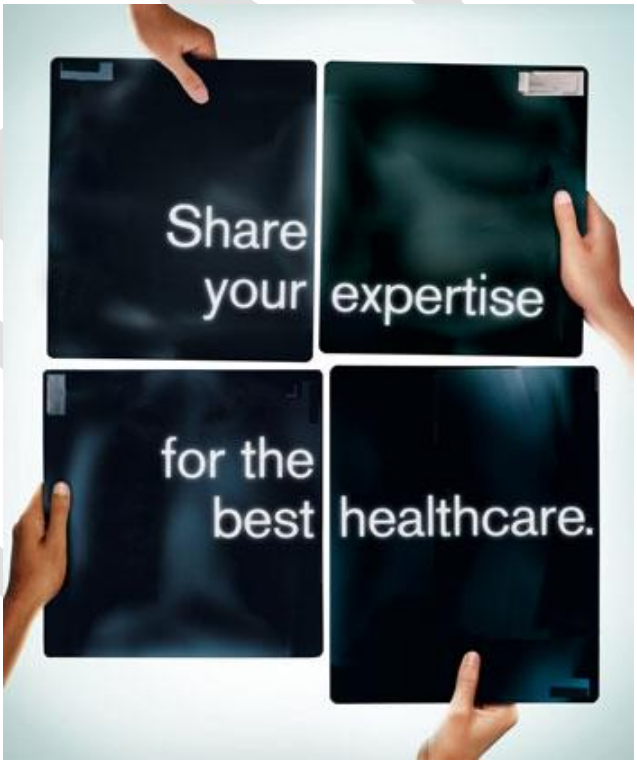
Literature Review part II

Specialised Healthcare Services and Networks



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Literature review part II: on Specialised Healthcare Services and Networks

1. Executive Summary

1.1 Introduction

This literature review has been conducted by the Partnership for Assessment of Clinical Excellence in European Reference Network (PACE-ERN) consortium, under the framework of a contract with the European Commission to develop a technical proposal for an Assessment Manual and Toolbox (AMT) for European Reference Networks (ERNs).

This report focuses on a review of a specialised health services and networks and is a complement to the literature review (part I) on best practices for developing an assessment program for health care services.

1.2 Objectives

The objectives of the literature review are to compile and analyse the information available related to the development, implementation and evaluation of specialised health care providers and networks in the area of rare or low prevalence and complex diseases.

1.3 Methods

A comprehensive literature review was completed based on the Arksey and O'Malley methodological framework. The model consists of identifying a plan for the literature review that specifies the sources to search, the keywords to use, and timeframes. To complete the literature review, sources were consulted between February and April 2015.

1.4 Results

The results from the literature review highlighted best practices and key considerations for the development of the assessment program for ERNs. It includes the variability amongst the assessment methods of specialised health care providers across Europe. Some member states have adopted a broader approach for designating specialised centres of expertise while others focused on rare diseases in general or a specific group of rare diseases. There are learning opportunities from pilot ERNs, informal networks for rare diseases across Europe, and mature networks such as trauma. The results also highlighted best practices that would help raise the bar for ERNs beyond the requirements in the Delegated and Implementing Decisions. Examples include forming international collaborations, and co-designing networks with patients and their families.

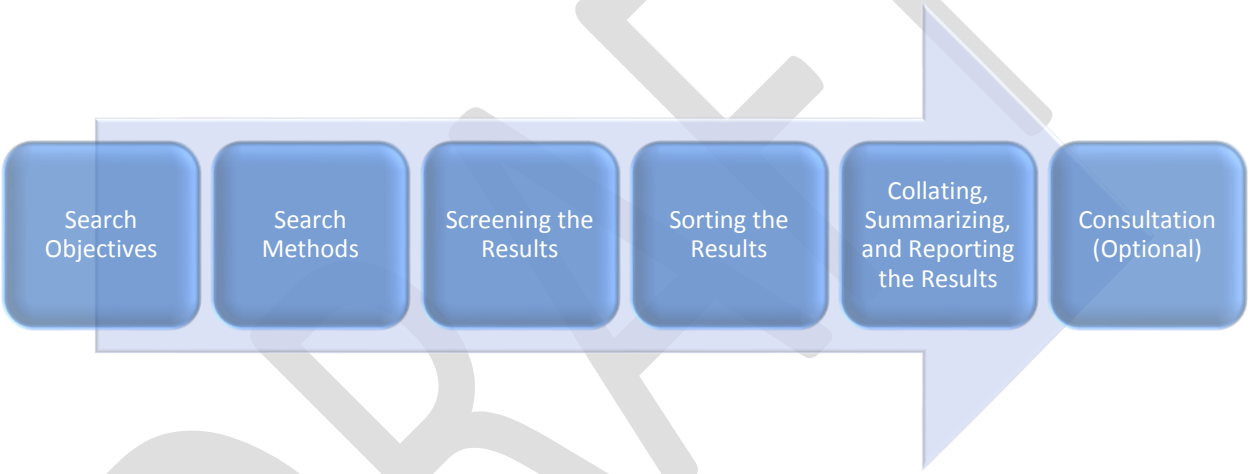
2. Introduction

The European Commission (EC) estimates that there are between 5000 and 8000 distinct rare diseases affecting more than 25 million people in Europe. A disease is considered rare by the EC if it affects fewer than 5 people in 10 000. The literature review compiled and analysed the information available related to the assessment of centres of expertise and networks in the area of rare or low prevalence and complex diseases. The results from the literature review are summarized in this report.

3. Methods

3.1 Model

The comprehensive literature review is based on the Arksey and O'Malley methodological framework. The following is an overview of the six-stage model:



3.2 Search Objectives

The purpose of the literature review is to identify information related to the assessment of centres of expertise and networks in rare or low prevalence and complex diseases. This is a summary of the research questions that were explored during the literature review:

- What are the processes used to evaluate centres of expertise in rare or low prevalence and complex diseases?
- Are there networks of centres of expertise in rare or low prevalence and complex diseases? If yes, how are they evaluated?
- What are the processes used to evaluate networks of centres of expertise in some of the common diseases and injuries (e.g. trauma)? Are there transferrable elements that could help in the assessment of ERNs?
- What are the specific recommendations for ERNs from projects financed by the EC?
- What other information from scientific publications can be used to inform the development of the ERN assessment program?

3.3 Search Methods

To complete the literature review, the following sources were consulted between February and April 2015:

- Electronic databases (open / closed)
- Reference lists
- Scientific publications
- Grey literature
- Relevant organisations and conferences
- Projects financed by the EC

The literature review was conducted using the following keywords, and inclusion and exclusion criteria.

Categories	Keywords	Inclusion / Exclusion Criteria
Clinical Area	▪ Rare or Low Prevalence and Complex Diseases / Conditions ▪ Highly Specialized Disease / Condition ▪ Low Prevalence ▪ Orphan Disease / Condition	***Inclusion Criteria:*** ▪ English language documents ▪ Public and private organizations ▪ Accreditation, licensing and certification bodies ▪ International sources ***Exclusion Criteria:*** ▪ Documents in other languages due to the cost and time involved in translating materials ▪ Documents published +15 years ago as the intent of the literature review is to reflect the current landscape of the assessment of networks and centres of expertise
Assessment Process	▪ Certification ▪ Accreditation ▪ Assessment ▪ Licensing ▪ Evaluation ▪ Audit ▪ Public / Private ▪ Centres of Expertise / Health Care Providers ▪ Networks / Groups ▪ Surveyor Model ▪ Decision Guidelines	
Assessment Requirements	▪ Standards ▪ Clinical Practice Guidelines ▪ Best Practices ▪ Evidence-based Guidelines ▪ Performance Indicators ▪ Assessment Manuals ▪ Tools / Technical Documents ▪ Associations	
EC Regulations	▪ European Reference Networks ▪ EC Reports ▪ EC Directives	

Search strategies with electronic databases included a combination of keywords and subject headings to be as exhaustive as possible. A list of subject headings was used to search key concepts in the CINAHL, Medline, PubMed, Cochrane and Joanna Briggs Collaboration. The search was iterative in nature and as the key concepts were searched, they were supplemented with keyword searching. No limitations were placed on study designs.

The search terms were used to attempt to limit literature results regarding the etiology of rare diseases since they were not the focus of this search. The search included works from the years 2000 to 2015 (current), English language and human studies. In some search engines the term 'rare disease' yielded more initial results versus the use of the term 'orphan disease'.

The list of inclusion and exclusion criteria was revised based on initial findings, and increasing familiarity with the topic being researched. As information on assessment programs for networks of centres of expertise in rare or low prevalence and complex diseases is scarce, the focus of the literature review expanded to include networks of common diseases and injuries. This search was focused on examples of mature networks such as trauma, stroke and cancer care.

Direct searches were also completed of assessment bodies at the European and international level to gather information related to centres of expertise and networks in the area of rare or low prevalence and complex diseases. The environmental scan focused on identifying existing processes for the evaluation of centres of expertise and networks in rare or low prevalence and complex diseases. The exhaustive review and on-site visits to the 10 member states, and the consultation questionnaire results will provide a more detailed assessment of the various stages across Europe with respect to developing a program for evaluating centres of expertise and networks in the area of rare or low prevalence and complex diseases.

3.4 Screening the Results

The inclusion and exclusion criteria were applied to screen all the documents resulting from the initial literature review. Full articles were obtained for studies that seem to be in line with the search objectives based on their abstracts.

The CINAHL database provided few results for examination, 3 articles were retained and upon further review were discarded. The Medline database resulted in 6 potential articles for possible inclusion. Upon closer examination all but one of the articles were discarded for being off topic or not meeting the set inclusion criteria. The PubMed database produced the largest number of potential results with 129 articles. After reviewing the titles and abstracts, 11 of these articles were examined more closely and seven were retained for review. The Cochrane database yielded a potential of 6 systematic reviews, however upon closer examination they did not meet the inclusion criteria. The Joanna Briggs Collaboration database yielded one potential systematic review, but upon examination it did not meet the inclusion criteria and was discarded. A total of 8 articles were retained from the combined database search for the purpose of full review and data extraction.

As this search strategy yielded few results, the search methods were expanded to include Google Scholar to obtain additional sources of information related to the assessment of centres of expertise and networks. This broader strategy helped identify a list of informative websites such as associations in the area of rare diseases that were further explored during the literature review.

3.5 Sorting the Results

In line with the search objectives, the information obtained from the literature review was grouped into the following categories:

- Assessment of Centres of Expertise in Rare or Low Prevalence and Complex Diseases
- Assessment of Networks:
    - Rare or Low Prevalence and Complex Diseases
    - Common Diseases and Injuries
- Recommendations for ERNs

3.6 Collating, Summarizing, and Reporting the Results

Accreditation Europe used the above framework to categorize and present the findings from the literature review. In summary, the literature review report includes the following information:

- An overview of the literature review methodology
- A summary of key elements related to the assessment of centres of expertise and networks
- Recommendations to consider during the development of the assessment program for ERNs

4. Results

4.1 Assessment of Centres of Expertise in Rare or Low Prevalence and Complex Diseases

The European Commission (EC) has led a number of collaborative initiatives to raise the profile of rare diseases under the guidance of experts from the field consisting of the Rare Diseases Task Force (2004-2009), the European Union Committee of Experts on Rare Diseases (2010-2013), and the EC Expert Group on Rare Diseases (2014-Present).

The Rare Diseases Task Force (RDTF) worked with the EC to develop a European strategy for rare diseases as outlined in the report called Rare Diseases: Europe's Challenges (2008). This work led to the adoption of the Council Recommendation on European Action in the field of Rare Diseases by the European Union Council in 2009. One of the key recommendations was for the member states to develop national plans or strategies for rare diseases by the end of 2013.

To support this work, the EC funded the European Project for Rare Diseases National Plans Development (EUROPLAN) to provide guidance on the development, implementation and evaluation of national plans or strategies for rare diseases, including the use of indicators (2008-2011). The recommendation was to

develop plans or strategies that cover the following themes: research, clinical care, orphan medications, social services, surveillance, and indicators. The plans or strategies would also ensure the sustainability of centres of expertise over time. The EUCERD Recommendations on Core Indicators for Rare Disease National Plans/Strategies (2013) outlined 21 core indicators to monitor the implementation of the national plans or strategies (e.g. number of centres of expertise adhering to national policy and participating in ERNs).

The European Union Committee of Experts on Rare Diseases (EUCERD) continued this work through the EUCERD Joint Action: Working for Rare Diseases initiative in 2012. The Committee provided recommendations to help provide quality care to patients with rare diseases through the following activities:

- Implementation of national plans or strategies for rare diseases
- Standardisation of the terminology used for rare diseases at the international level
- Mapping the provision of specialised social services and integration of rare diseases into mainstream social policies and services
- Leveraging of the value of networking for improving the quality of care for rare diseases at the European level
- Integration of rare disease initiatives at the national level across Europe

These drivers led member states to develop national plans or strategies for rare diseases, and assessment processes for designating centres of expertise. Centres of expertise are defined as "expert structures for the management and care of rare disease patients" (EUCERD, 2011). These facilities are often research or teaching hospitals, with highly specialized professionals in the field. Centres of expertise are described as the key elements of European Reference Networks (ERNs). It is the links between centres of expertise and other organizations that allow for knowledge and resource sharing, bringing specialized health services to the patient's local community. Some countries have also established centres of expertise outside the field of rare diseases. Centres of expertise need to be regularly evaluated in order to ensure the integrity of their designation within a member state.

The member states are at different stages of development and implementation of these initiatives across Europe. In some countries, this work had started before the Council Recommendation of 2009. As a result, the assessment processes for centres of expertise in rare or low prevalence and complex diseases vary significantly across Europe. There are also assessment processes established for centres of expertise and networks for other types of diseases such as cancer care.

An environmental scan has been completed to identify existing assessment processes to evaluate centres of expertise in rare or low prevalence and complex diseases. The focus is on member states with a formal designation program for centres of expertise. A more detailed evaluation of the European landscape that includes informal designation processes for centres of expertise (e.g. self-declaration) will be captured as part of the exhaustive review, on-site visits with the member states, and the consultation questionnaire. The objective of the literature review is to identify best practices for

assessing centres of expertise and networks. The other mapping activities will provide a broader picture of the current landscape of centres of expertise and networks across Europe.

4.1.1 Belgium

In Belgium, the National Plan for Rare Diseases promotes the development of centres of expertise for rare diseases and outlines requirements in line with the EC recommendations. The following is a summary of these requirements:

- Developing, implementing and sharing clinical practice guidelines
- Submitting patient information to a national registry
- Creating networks with national and European health care providers
- Participating in training and research activities
- Collaborating with patient organizations and the media
- Transferring patients to another centre of expertise across Europe (as needed)

Centres of expertise are currently accredited under the health insurance system in Belgium. The Plan recommends having a separate accreditation process for centres of expertise based on the requirements outlined in the Plan. Next steps include developing a framework for networks of centres of expertise.

4.1.2 Denmark

In Denmark, the Danish Health and Medicines Authority is responsible for designating centres of expertise (known as 'highly specialised functions') in line with the Health Care Act. These include two designated centres of expertise for rare diseases established in 2001, and referral centres of expertise in a specific or several specific rare diseases. The National Plan from the National Board of Health reports approximately 100-120 designated centres in the area of rare diseases. The designation is renewed every 3 years. The following is a summary of the criteria applied to establish a centre of expertise:

- Rareness and complexity of the disease
- Multidisciplinary approach needed
- Expensive technology required for diagnosis and treatment

4.1.3 France

In France, the National Plan for Rare Diseases outlines a designation process for centres of expertise in the area of rare diseases (known as 'reference centres'). There are 131 designated centres of expertise with an assigned coordinator in each centre and dedicated funding. The following is a summary of the requirements for centres of expertise:

- Provide expertise in one or several rare diseases as designated, and direct patient care
- Working with the French National Authority for Health (HAS), contribute to clinical practice guidelines for rare diseases at the national level

- In collaboration with the French Institute for Public Health Surveillance, participate in research activities related to the epidemiological surveillance of rare diseases
- In collaboration with the French national Institute for Prevention and Health Education, participate in education and training activities for health care professionals, and patients and their families
- Coordinate networks of health care professionals and social workers
- Act as a liaison for patient organisations and social workers

Each centre of expertise is linked to approximately 500 regional centres per region (known as 'competence centres'). These regional centres are designated by the French Regional Hospital Agencies, and assist centres of expertise by providing services closer to the patient's home. Services relate to the diagnosis, treatment and follow-up of patients with rare diseases. These centres do not receive dedicated funding.

The Ministry of Health is working on a framework to group centres of expertise. The 131 centres of expertise in France could potentially be grouped into 23 networks. Next steps include broadening the scope of networks of centres of expertise to include key stakeholders such as regional centres, diagnostic and research laboratories, social care services, and patient organizations. These organizations would be designated as a network and receive dedicated funding from the Ministry of Health. A process is being developed for the renewal of centres of expertise in general. It is not specific to centres of expertise in rare diseases.

4.1.4 Greece

In Greece, the Ministry of Health can designate centres of expertise (known as 'specialised reference centres') in line with the National Health System Act. There are no specific requirements or criteria for these centres of expertise. Applications are initially reviewed by the Central Council of Health and then approved by The Ministry of Health. There are a number of centres of expertise in rare diseases that are not designated under this process.

Next steps are to establish a formal framework for designating centres of expertise in rare diseases that outlines specific criteria and requirements. This work is undertaken by an Advisory Committee on Rare Disorders in collaboration with the Hellenic Centre for Disease Control and Prevention.

4.1.5 Italy

In Italy, the National Centre for Rare Diseases of the National Health Institute provides oversight to networks of centres of expertise in rare diseases (known as 'certified centres'). The following is a summary of the requirements for interregional centres in the network:

- Sharing information related to rare diseases
- Coordinating care across the centres of expertise in the network to endure timely access to services

- Sharing information related to the availability of medication to patient organisations, and patients and their families

Rete Nazionale Malattie Rare is the first example of a network of centres of expertise responsible for providing care related to rare diseases. The centres of expertise have been designated by regional health authorities to diagnose and treat rare diseases in line with pre-determined clinical practice guidelines. Other requirements include participating in prevention activities, surveillance, knowledge exchange, and opportunities to improve diagnosis and treatments. These centres also work with the patient's primary health care provider to coordinate care.

There is also an accreditation model for haemophilia centres in Italy developed by the Italian Association of haemophilia centres. The recommendation was to implement this model across 21 regions to provide patients with haemophilia and allied inherited coagulations disorders access to quality standardised care. The requirements address medical records, patient safety, treatment plans, home care, regular follow-ups, access to laboratories, education and training, and quality improvement. A study by Mannucci et al. (2014) examined the accreditation model for haemophilia centres. It includes completing a self-assessment against the standards consisting of clinical and organizational requirements followed by an on-site visit by peer reviewers. The results of the study indicated that the centres of expertise performed better on clinical rather than organizational requirements.

In this same perspective, another article by Giangrande et al. (2014) highlighted the European standards of haemophilia centres. In particular it notes that there are many health care providers that have labelled themselves as haemophilia centres despite their size, expertise and services provided. The authors note that the implementation of European Guidelines for the certification of haemophilia centres will standardise quality in this sector and reduce health inequalities. They also suggest that this model could be utilized for other groups of rare diseases.

4.1.6 Netherlands

In the Netherlands, centres of expertise (known as 'specialised centres') are governed by the 1997 Special Medical Procedures Act through the Ministry of Health, Welfare and Sports. A threshold is applied to determine if a hospital can provide care for rare diseases. For example, the Dutch Health Care Inspectorate requires that hospitals perform a minimum of 20 oesophagus cardia resections per year to be able to continue to provide this treatment.

There are plans to provide a framework for networks that would involve university medical centres and general hospitals. Some of the requirements for these networks would include:

- Participation in clinical studies and contribution to research in specific diseases
- Contribution to national and international knowledge exchange with other centres of expertise, smaller hospitals and primary care centres

Patient organisations also seem to play a role in providing resources for health care providers. Alliance for Heredity Issues is an alliance of several patient organizations with a genetic, congenital or rare

disease. The group actively supports centres of expertise by developing guidelines and criteria for multidisciplinary care.

There is also certification model for haemophilia centres based on the standards endorsed by the Central body of Experts on quality standards in clinical care and the Ministry of Health. Haemophilia centres are certified based on results from an on-site visit completed by a team of haemophilia clinicians, nurses and an auditor. Certifications are reviewed annually with on-site visits every three years.

4.1.7 Norway

In Norway, there are 10 centres of expertise in rare diseases (known as 'specialist health care services') designated by the South Eastern Norway Regional Health Authority with dedicated funding. These centres offer services in more than 350 different rare diseases. Centres of expertise are designated if the disease is sufficiently complex, and requires access to multidisciplinary care and services from multiple health care providers. These centres of expertise are also responsible for knowledge exchange.

4.1.8 Spain

In Spain, centres of expertise (known as 'reference centres') are designated in line with the Spanish National Health Service Cohesion and Quality Act. The designation process involves a preliminary review of the centre of expertise by the Designation Committee to confirm eligibility. The centre of expertise is then evaluated by the Quality Agency against a set of requirements. Successful centres are designated by the Ministry of Health, Social Policy and Equity for five years. To maintain the designation, a reassessment is required by the Quality Agency. The following is a summary of the requirements for centres of expertise:

- Equitable access
- Patient safety
- Quality improvement
- Effectiveness of diagnosis and treatments
- Efficiency of services
- Sustainability
- Training and research experience
- Multidisciplinary approach
- Appropriate resources
- Performance indicators
- Information systems

4.1.9 Sweden

In Sweden, the National Board of Health and Welfare is responsible for designating centres of expertise (known as 'providers of specialist care') in line with the 1990 agreement with the Federation of County

Councils. The centres for rare diseases are primarily located at the university medical centres. The designation process requires that centres of expertise are endorsed by a country council. The following is a summary of the requirements evaluated by the National Board of Health and Welfare:

- Demonstrate competence in the field
- Provide quality care through patient safety and quality improvement programs
- Demonstrate business continuity
- Ability to expand scope of activities based on patient needs

4.1.10 United Kingdom

In the United Kingdom (UK), centres of expertise (known as 'specialist centres') are designated through the National Health Service (NHS) commissioning process. The following is a summary of the requirements for centres of expertise:

- Providing quality care
- Adopting financial and clinical standards
- Sustainability of skills, experience and resources
- Demonstrating excellence and innovation
- Adopting quality standards and measurable outcomes
- Developing a quality improvement program
- Sharing information with the public about designation
- Collaborating with professional and patient groups (if available)
- Demonstrating that services are more effective than other similar services in the field

Successful applicants are designated as centres of expertise at the national level. Designation is renewed by the Secretary of State upon recommendation from the Advisory Group for National Specialised Services.

4.1.11 International

Europe is a pioneer in the area of establishing centres of expertise and networks specifically in the area of rare or low prevalence and complex diseases. Other countries such as Canada and Australia are in the preliminary stages of identifying recommendations for supporting patients affected by rare diseases. For example, in Australia, the following strategies are being explored:

- Equitable access to care
- An overall model for providing care for patients with rare diseases
- Coordinated care across the patient's life-span
- Multidisciplinary approach that includes social services
- Early diagnosis
- Communication and information sharing
- Promoting existing support groups and/or creating new ones as needed

In Canada, similar goals were established for patients with rare diseases including public awareness, recognition, prevention and early detection, community resources, timely and equitable care, informed decisions, sustainable access, and innovative research.

4.2 Assessment of Networks

4.2.1 Rare or Low Prevalence and Complex Diseases

There is overall support from the literature for creating networks of centres of expertise across Europe. An article by Taruscio et al. (2014) focuses on the collaborative efforts of the EUCERD and the EC in the field of rare diseases. The authors discuss the EUCERD Recommendations on Centres of Expertise and on European Reference Networks. They conclude that centres of expertise have the potential to facilitate disease management and improve patient care. Financial support and evaluation of these centres are key elements that require oversight.

However, there is variability in how networks are formed across Europe. Some of the member states grouped highly specialised services in a small number of centres of expertise. These centres of expertise are not specific to rare or low prevalence and complex diseases. Other member states took a different approach where centres of expertise are specifically designated to provide care to patients with rare diseases in general, or a specific group of rare diseases. This method centralises efforts to research diagnosis and treatments in a specific area of rare or low prevalence and complex diseases. At the European level, there are some informal cross-border networks that provide lessons learned to help structure the evaluation of ERNs in the future. The following is a summary of these networks.

4.2.1.1 Pilot ERNs

Over the last years, while the policy and legal framework for ERNs was being developed and consolidated, the EC supported informal networks in rare diseases. The following is the list of the 10 pilot ERNs for rare or low prevalence and complex diseases financed by the EC:

- Dyscerne: European Network of Centres of Reference for Dysmorphology
- ECORN CF: European Centres of Reference Network for Cystic Fibrosis
- PAAIR: Patient Associations and Alpha1 International Registry (PAAIR)
- EPNET: European Porphyria Network - providing better health care for patients and their families
- EN-RBD: Establishment of a European Network of Rare Bleeding Disorders
- Paediatric Hodgkins Lymphoma Network: European-wide organisation of quality controlled treatment
- NEUROPED: European Network of Reference for Rare Paediatric Neurological Diseases (ended)
- EURO HISTIO NET: A reference network for Langerhans cell histiocytosis and associated syndrome in EU
- TAG: Improving Health Care and Social Support for Patients and Family affected by Severe Genodermatoses – TogetherAgainstGenodermatoses

- CARE NMD: Dissemination and Implementation of the Standards of Care for Duchene muscular Dystrophy in Europe (including Eastern countries)

The focus of these networks was to network, share expertise for treating and managing patients, implement standards of care, and contribute to research. The evaluation results for these pilots were summarized in the EUCERD Report: Preliminary analysis of the outcomes and experiences of pilot European Reference Networks for rare diseases (2011). The report recommended providing support to the following key resources for developing and implementing ERNs: shared patient registries and databases, tools for telemedicine, clinical practice guidelines, and education and training activities. The pilot also confirmed the importance of investing in telemedicine to make sure that the expertise travels rather than the patient. This is based on the Council Recommendations "In principle and where possible, expertise should travel rather than patients themselves" (2010).

The pilots have also shown that the composition of ERNs could go beyond centres of expertise to include laboratories and patient organisations. ERNs also differ in the range of services that they provide to patients with rare diseases. They could be a basic research network, a clinical care network, an information network for patients and the public, or a comprehensive network that delivers a mix of research and clinical care.

The report also highlighted other recommendations including the need to foster and designate expertise at the national level as a first step towards forming ERNs. There also needs to be dedicated resources to support ERNs that include having a coordinator responsible for managing activities across the network with funding provided at the European level. There also needs to be a quality improvement program in place to evaluate ERNs and address areas for improvement. Other considerations from the report include determining a process for supporting existing ERNs and promoting the creation of new ones moving forward. Challenges include prioritizing funding for ERNs and providing financial support for a period of 5 years.

In summary, the report highlighted the diversity of networks across Europe. The following is an overview of the findings:

- Variability in the geographic representation of member states where not all the countries are covered by ERNs
- Sustainability of networks and their funding
- Recommendations to ensure that expertise travels instead of the patient
- Ongoing evaluation of ERNs at the European level

4.2.1.2 European Haemophilia Network

The European Guidelines for the certification of haemophilia centres is a European initiative that is part of the European Haemophilia Network project. It is a voluntary certification program that is available to all haemophilia centres across Europe. It provides a concrete example of the application of the European Commission Cross-border Health Care Directive (article 12).

The European Guidelines for the certification of haemophilia centres consist of two categories of requirements. The first is focused on European Haemophilia Comprehensive Care Centres that provide complex services as tertiary referral Centres. The second is the European Haemophilia Treatment Centres that provide basic care. The following is a summary of the themes covered by this network:

- General requirements (e.g. policies and procedures, medical records and patient registries)
- Patient care (e.g. diagnosis and treatments)
- Advisory services
- Laboratory services
- Networking of clinical and specialised services
- External assessments for diagnostic tests
- Training and education
- Telemedicine
- Research in multi-centre settings
- Networking with centres of expertise
- Patient empowerment

Overall, there is alignment between these themes and the EC regulatory framework for ERNs.

The article from Calizzani et al. (2014) on the assessment methods for haemophilia centres recommends involving patients in the various stages of the process. There are various models to incorporate patient involvement either as a champion of the assessment process or as part of the assessment team that reviews the services provided by the centre of expertise.

4.2.1.3 European Network for Rare and Congenital Anaemias

The European Network for Rare and Congenital Anaemias (ENERCA) was launched with financial support by the EC. The objectives of ENERCA are to provide centralized information to the public, health care professionals and patients with rare and congenital anaemias. Over the years the mandate of ENERCA has expanded to include the application of the European Commission Cross-border Health Care Directive (article 12). ENERCA is working on forming linkages with the European centres of expertise in rare anaemias to provide resources and direct patient care. This will include an e-health platform for telemedicine, electronic health records and e-learning.

As part of this work, ENERCA published a report on the Recommendations for centres of expertise in rare anaemias: a white book (2014). It's the first report that provides multi-faceted requirements that centres of expertise in rare anaemias need to meet to join an ERN in this area. The following is a summary of the requirements:

- Adopt a multidisciplinary approach
- Provide expertise and resources
- Create linkages with other centres of expertise across Europe
- Create linkages with 'local centres' within a defined catchment area to coordinate care

The report also highlights challenges and recommendations for future ERNs in the area of rare anaemias:

- Communication channels and translational referral processes
- Transfer of patient information and samples, and in some cases patients
- Variability amongst national regulations (e.g. genetic counselling and testing)
- Identify consistent criteria for following protocols that are in line with national laws

An example of a different type of network is the EuroBioBank Network. An article by Mora et al. (2014) focuses on the operations of the EuroBioBank Network which is a group of biobanks in Europe, used to provide human samples to the scientific community conducting research on rare diseases. It serves as an example of a network that utilizes expertise, high professional standards and best practices. The network is implementing integration with rare disease patient registries in efforts to mitigate fragmentation of international synergies. This might be an opportunity to have an ERN that would consist of a mix of health care providers and laboratories.

4.2.1.4 Rare Cancers Networks

The European Commission has also supported another project under the Health Programme in the area of rare cancers. The Information Network on Rare Cancers (RARECARENET) is a project created in 2012 to build a network of centres of expertise in rare cancers to standardise care and facilitate access to quality services across Europe.

One of the main objectives of this project is to identify requirements for centres of expertise for rare cancers and share information on designated centres of expertise. The project is currently in progress. The following a summary of the themes covered:

- Promote a common classification of rare diseases
- Contribute to information available on rare cancers through publications
- Improve diagnosis, treatment and referral of patients with rare cancers to appropriate centres of expertise
- Promote European and international collaboration for researching rare diseases
- Identify and address barrier to ensure equitable access to care
- Invest in planning and resource allocation for rare cancers
- Foster a client-centred approach to care

4.3 Common Diseases & Injuries

The following is a select sample of assessment programs for common diseases such as trauma, stroke and cancer to identify transferrable recommendations for ERNs, as well as examples of cross-border initiatives across Europe.

4.3.1 Trauma

In Canada, the field is moving towards a network approach for trauma centres. The trauma network encompasses pre-hospital, inter-facility transport, trauma centres and rehabilitation services. These services are integrated within the trauma network to maximise the recovery of trauma patients. The rationale for this approach is that rehabilitation should start early within the trauma centre and continue once the trauma patient is transferred to a rehabilitation centre or discharged home. Trauma networks need to demonstrate coordination and leadership of the trauma network at the provincial, regional and/or sub-regional level. The configuration of the trauma network will depend on the size of the population and catchment area. For example, regional trauma networks are normally based on a population of 1 to 2 million people. These networks consist of one or two major trauma centres to handle severe and complex injuries (i.e. Level I or II) and a number of local hospitals identified as level III, IV or V trauma centres. These centres treat less severely injured patients or stabilise major trauma patients before transferring them to a Level I or II trauma centre.

Accreditation Canada collaborated with the Trauma Association of Canada in 2013 to transition the accreditation of trauma services and develop an assessment program for trauma networks. The program includes requirements at both the network and centre level where the majority of the requirements apply to the Level I and II trauma centres. The following is a summary of the requirements at the trauma network level:

- Establish an administrative structure (e.g. lead agency for the network, and medical and administrative leaders)
- Collect information on injury rates
- Contribute to targeted injury prevention programs and evaluate impact on injury rates
- Support trauma-related research activities
- Partner with other trauma networks across borders through intra- or inter-provincial/territorial agreements
- Invest in information systems including telemedicine
- Standardise protocols across the trauma network including the pre-hospital and rehabilitation networks
- Evaluate the trauma network on an ongoing basis and make improvements

One of the requirements is to have agreements in place to coordinate services that are not available within the trauma network. This includes access to paediatric trauma centres or specialised rehabilitation services.

A similar approach has been adopted through a bilateral agreement between the United Kingdom and Malta called the National Highly Specialized Referrals Programme which has been in place for over 60 years. This initiative is governed by the Malta–United Kingdom Health Care Agreement. Malta can send a fixed number of patients to the UK to receive specialised treatments not available in Malta for free. Malta is expanding this approach to operate patient referral programmes with a number of centres of expertise in Italy in the area of hepato-biliary services and lung transplantation.

4.3.2 Stroke

The North Central London Cardio Vascular & Stroke Network is an example of a regional stroke network in the UK. The network consists of health care providers from primary, secondary and tertiary care to ensure standardise care and coordinated services for stroke patients. Health care providers can be designated to operate hyper acute stroke units or acute stroke units. The process includes submitting an application to an expert panel organised by NHS London. The requirements include providing quality care, adopting a multidisciplinary approach and being located in an area where stroke coverage is needed.

An article by Podolec et al. (2014) focuses on the Centre for Rare Cardiovascular Diseases in Krakow, Poland to emphasize the importance of having a consistent language in the field. It is a designated centre of expertise for cardiovascular diseases. The authors note that patients often deal with inequities for access to their professional care. One barrier they note is the lack of a comprehensive classification for these diseases. The information from this article supports the importance of having common language and classification systems in order to reduce barriers and create a network that functions effectively.

4.3.3 Cancer

Another example of a cross-border initiative in Europe is the model adopted by Sweden and Denmark in the area of cancer care networks in 2001. The initiative is called the Joint Unit for Breast and Endocrine Surgery and is established between the University Hospital in Lund and Copenhagen University Hospital. The project facilitates access to quality care to patients requiring surgical treatment related to breast cancer.

The NHS Cancer Programme in the UK designates eligible hospitals to provide specialised treatments for different cancers. These cancer networks were set up in 2000 to provide coordinated care for cancer patients. The designation is done through committees consisting of experts in the field. Committee members also contribute to the development of clinical practice guidelines for various groups of cancers.

Another example is the Czech Cancer Centre Network initiative led by the Czech Society for Oncology since 2006. The Society has designated a network of 18 centres of expertise in the area of diagnosis and treatment of cancer tumours (known as ‘comprehensive cancer centre’). The number of eligible centres has been reduced to 13 following an audit conducted by the Ministry of Health in 2008. Requirements included ensuring expertise in various cancer diagnoses, and providing access to palliative care as needed.

4.4 Commission’s Advisory expert groups and recommendations

The European Commission had led a number of activities on the topic of Centres of Expertise and Networks in the field of rare diseases (Rare diseases expert group of national authorities, independent experts and stakeholders).

4.4.1 Centres of Expertise Reports

The following is a summary of the recommendations from EUCERD and RDTF reports that provides guidance on the key features to be considered when developing an assessment program for ERNs. Examples include the RDTF Report: Overview of Current Centres of Reference on rare diseases in the EU (2005), RDTF Report: Centres of Reference for Rare Diseases in Europe (2006), and RDTF Report: Recommendations of the Rare Diseases Task Force (2006).

Overall, these reports indicated that the member states are at different stages of developing an assessment process for centres of expertise for rare diseases. They recommend that centres of expertise and networks cover the following key themes:

- Appropriate and effective resources to diagnose, treat and manage patients with rare diseases
- Quality services
- Capacity to provide expert advice (e.g. confirmation of diagnosis)
- Implement clinical practice guidelines
- Track outcome indicators
- Multi-disciplinary approach
- Contributions to research through grants and publications
- Teaching and training activities
- Participation in epidemiological surveillance (e.g. patient registries)
- Collaboration with other centres of expertise at the national and international levels
- Collaboration with patients associations (if applicable)

4.4.2 National designation of centres of expertise

The EUCERD Workshop Report: National Centres of Expertise for Rare Diseases & European Collaboration (2011) highlighted that one of the critical requirements for implementing the Cross-Border Health Care Directive is to have a consistent approach for designating centres of expertise across Europe. Having similar requirements or criteria for centres of expertise helps ensure a similar level of quality of care across the network. Member states with no current assessment processes are recommended to develop a designation policy based on learnings and shared experiences from other countries. An alternative would be to set up agreements with other member states to use services from their centres of expertise in rare or low prevalence and complex diseases. There may need to be a combination of both options as not all rare diseases could be covered at the national level.

4.4.3 Patient organisations and patient engagement

Most member states across Europe have National Alliances that serve to federate patient organizations within a particular country. Thirty-two of these National Alliances are member of The European Organisation for Rare Disorders (EURORDIS). This umbrella organization represents 614 rare disease patient organizations throughout fifty-eight countries. It can therefore provide for rare disease advocacy at the European level. Similarly, the Canadian Organization for Rare Disorders (CORD) represents a

collective of organizations that advocate for and support those living with rare disorders. This strengthens the position of these organizations in order to influence health policy and systems reform. Individually, these organizations may lack the resources to act at this level (CORD, 2014). CORD is currently looking at developing plans and strategies for rare diseases.

Patient engagement is one of the key themes highlighted from the literature review. Several articles have been published on the concept of patient- and family- centred care. For example, an article from Bate et al. (2006) promotes the redesigning of the health care system from around the patient to co-designing services with the patient. This is an emerging and innovative concept for consideration when developing an assessment program for ERNs.

This concept is in line with the EURODIS Declaration of Common Principles on Centres of Expertise and European Reference Networks for Rare Diseases (2008). These principles include specific recommendations for involving patients in the centres of expertise and networks in rare diseases, including:

- Providing a comfortable and safe environment for patients
- Providing access to centres of expertise to all patients across Europe
- Involving patients in the development, implementation and evaluation of the centre and network and sharing the results publically
- Expanding education and training activities to health care professionals as well as patients
- Recognising patients are as active partners and involving them at all stages of their care

These recommendations are also in line with the Patients 'Consensus on Preferred Policy Scenarii for Rare Diseases (Polka) project which was an initiative led by EURODIS and co-financed by the EU Public Health Programme (2008-2013). The purpose of this project was to obtain direct patient input on the plans and strategies for rare diseases at the European level.

4.4.4 Patient Registries and Databases

The Council of the European Union Recommendation on Rare Diseases (2009) supported the development of patient registers and databases in the area of rare or low prevalence and complex diseases. This initiative would facilitate access to the information needed to conduct research in the field. Some of the challenges involve protecting personal information at the European Union level.

4.4.5 International Collaboration

The EC is increasingly promoting international initiatives in the area of rare diseases. A key example is Orphanet created in 1997. It is a portal for information on rare diseases and orphan medications. Orphanet is also working with the Topic Advisory Group for Rare Diseases of the World Health Organization to review the international classification of diseases.

Another key international initiative led by the EC is the International Consortium for Rare Disease Research (IRDiRC) launched in 2010. Countries include the United States, Canada, and Japan. The

objective is to centralise and share best practice information related to diagnostic and treatment tools in the field. The goal is to have 200 new interventions for rare diseases by 2020.

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