



European
Commission

2nd CONFERENCE ON



European
Reference
Networks

CONFERENCE REPORT

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**8-9 OCTOBER
2015, LISBON**



GOVERNO DE
PORTUGAL

MINISTÉRIO DA SAÚDE

Presidency of
the Council of the
European Union

GRAND DUCHY OF

 Luxembourg

Health

European Reference Networks – 2nd Conference – Lisbon, 8 & -9 October 2015

Executive Summary

Health professionals, researchers, patient organisations and policymakers gathered in Lisbon on 8 & 9 October for the 2nd European Reference Networks Conference. The well-attended and lively event, for which 475 people registered, featured a full day of presentations and debate, followed by four parallel workshops on the second day. Delegates took advantage of networking opportunities throughout the conference, which was also streamed live online.

The event was timely. The first call for ERNs is expected in early 2016 and interested parties are already preparing for the call. The conference built on the 2014 event and was designed to offer practical guidance to individuals and organisations interested in achieving ERN status.

Opening the event, Xavier Prats Monné, Director General of DG SANTE, emphasised the value of the ERNs and the imperative of turning a “beautiful idea” into reality. He said ERNs are now embarking on a new journey and acknowledged the significant contribution the healthcare community has made along the road.

The first roundtable session explored the timetable and milestones for creating the ERNs and walked participants through the approval process that will follow first call. The key players were introduced, including the Board of Member States, describing the process for establishing ERNs. A second roundtable heard from health ministries in several Member States on the strategic value of ERNs and this was echoed by Ministers of Health for Portugal and Luxembourg. This political endorsement offered encouragement to those contemplating the significant task of building a network and making a submission next year.

A third session featured contributions from patient groups and coordinators of existing pilot networks funded under EU programmes. Their experiences and insights offered practical assistance as well as highlighting some of the challenges of establishing governance criteria and managing multi-partner cross-border networks. It also reinforced the potential value of ERNs in improving access to specialist services for patients and in ironing out inequalities across the EU. Session four explored the potential for ERNs to devise and implement clinical guidelines, before the fifth session examined the power and pitfalls of developing IT solutions for ERNs. Delegates saw a concrete example of how telemedicine tools can allow experts to review clinical cases online at any time and from any location. The importance of interoperability between IT solutions was also highlighted.

On day two, an opening plenary session offered practical tips on applying for ERN status. The group was then divided into four workshops, each examining a different element of building an ERN proposal. Valuable feedback was received from delegates and a number of points of clarification were sought on issues such as the financial sustainability of ERNs, the scope and likely number of networks, securing the endorsement of Member States, how to find out about ERNs that are in gestation in order to avoid duplication of effort, and mechanisms for sharing experience between ERNs once they are up and running. Patient-centricity was stressed throughout.

Introduction

The Directive 2011/24/EU on the application of patients’ rights in cross-border healthcare strengthens co-operation between highly specialised healthcare providers across the European Union by the establishment of a system of European Reference Networks (ERNs). The legal framework of ERNs adopted by the Commission entered into force on 27 May, 2014, after an exhaustive consultation process with national authorities, experts, and stakeholders.

ERNs will help to provide affordable, high-quality and cost-effective healthcare to patients with conditions requiring a particular concentration of resources or expertise. They will strive to improve access to the best possible expertise and care available in the EU for patients with rare diseases and complex conditions.

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Opening the event, Xavier Prats Monné, Director General of DG SANTE, emphasised the value of the ERNs and the imperative of turning a “beautiful idea” into reality. He acknowledged the significant contribution the healthcare community has made along the road and noted that ERNs are now embarking on a new journey. “We are here to help you prepare for the call that will come next year and to hear your concerns and suggestions on how to get it right,” he said.

Roundtable I: Framework for the establishment of European Reference Networks

Chair: Andrzej Rys; Director Health Systems and Products; DG SANTE

Enrique Terol, DG SANTE, outlined the milestones on the road to creating ERNs, beginning with free movement of patients in 2002. Following the implementation of the 2011 Cross-Border Healthcare Directive and the Commission decision on ERNs in 2014, the legal basis now exists on which to build networks that will add real value for patients with rare and complex conditions.

Dr Terol emphasised that ERNs would bring a multidisciplinary approach to healthcare in areas where there is a scarcity of knowledge and where investment by individual Member States would be insufficient in isolation. They have the potential to give patients across the EU access to the quality care which might otherwise not be available to them and to raise standards by developing guidelines and patient pathways for rare diseases.

He set out the criteria to be met by networks and each of their members in order to be awarded ERN status. Each ERN should appoint a coordinator who will put together the application and liaise with each of the Healthcare Providers participating in networks. All participants need the endorsement of their Member State.

“So far, more than 50 interested groups or pilot networks have been identified and we expect some very good proposals when the call is published in a few months’ time,” said Dr Terol. He acknowledged that the networks have, until now, been a little fragmented but several are now working to build strong applications in their areas of expertise. In most cases, patient and professional organisations are playing an active role.

A number of challenges lie ahead. These include:

- Attracting and approving the right ERNs
- Developing an effective network model and useful platforms/tools
- Securing Member State commitment and ensuring financial sustainability
- Avoiding fragmentation and overlap of ERNs
- Developing and deploying standardised tools such as clinical guidelines, registries, patient pathways and interoperable IT systems.

Key features of ERNs

- Patient-centred and clinically led
- 10 members in at least 8 countries
- Strong independent assessment
- Fulfilment of Network and Members criteria
- Endorsement and approval by national authorities.

‘IMAGINE IF THE BEST SPECIALISTS FROM ACROSS EUROPE COULD JOIN THEIR EFFORTS TO TACKLE COMPLEX OR RARE MEDICAL CONDITIONS THAT REQUIRE HIGHLY SPECIALISED HEALTHCARE AND A CONCENTRATION OF KNOWLEDGE AND RESOURCES’

In preparing an ERN application, it will be essential to convey the scope of the network to be addressed, the value a network would add, the players that will form the network and how the ERN and its members will fulfil the various criteria. To support applications, an Assessment Manual and Technical Toolbox have been developed to offer guidance for applicants and assessment bodies. A competitive call is currently under way to select Independent Assessment Bodies which will evaluate submissions for ERN status and make recommendations to the Board of Member States.

Looking ahead, after the first call is issued in first quarter of 2016, proposals will be assessed in the 2nd quarter of the year before the first ERNs are established in the 3rd quarter of 2016.

Matt Johnson, PACE-ERN Consortium, took participants through the Assessment Manual and Technical Toolbox of ERNs. He reiterated some of the minimum requirement for ERNs and explained how the Assessment Manuals and Technical Toolbox, for Applicants and for Independent Assessment Bodies were developed. These documents set out a range of operational criteria that Networks and Healthcare Providers must meet.

The road to becoming an ERN follows a six-stage process beginning with the call for interest by the European Commission early in 2016. Applicants will need to be 'endorsed' by their Member State and should perform a self-assessment using the criteria provided. An Independent Assessment Body (IAB) will review applications and make a recommendation to the Board of Member States (BoMS).

Till Voigtländer, Co-chair BoMS, provided further detail on the journey that networks must make in order to achieve ERN status and he introduced the BoMS. The BoMS is composed of representatives appointed from 30 EU and EEA Member States. Decisions will be made by consensus or, when this not possible, by a two thirds majority. Each country has only one vote. The primary role of the BoMS will be the approval or rejection on network proposals and healthcare providers' membership. It will also decide on whether healthcare providers can join existing networks. The Board has the power to terminate a network or a member of a network. Where negative assessments are made, the BoMS has the option of allowing a network or member to continue for one year based on an 'improvement plan', after which a re-evaluation will take place. In any case, all ERNs will be reviewed after five years according to the legal base.

Discussion

There was some debate about the role of research in ERNs. By definition, it was noted, ERNs should be patient-centred and focused on healthcare delivery. While research can flourish thanks to the networks of providers and patients connected through ERNs, the networks are not primarily research consortia. "We want to ensure that ERNs are not just research networks; the patient is central. We expect that proposals may contain a research component but there is a need for a strong clinical component," said Dr Voigtländer.

On the same theme, a question was asked about the link between ERNs and Horizon 2020 funding. "Is it necessary to be part of a network to apply for Horizon?" asked a health researcher. Enrique Terol explained that ERNs and Horizon 2020 are two quite different initiatives. Membership of an ERN could be considered an asset by Horizon evaluators, but it is not a requirement for securing EU funding from health or research programmes.

Several questions throughout the conference focused on the scope of ERNs: how broad or narrow should they be when defining their area of expertise? And how many networks will there be? It was noted that, legally, there is no limit to the number of ERNs that could be established, but that the Commission Expert Group on Rare Diseases suggested in its recently published opinion that 20-23 groups would be optimal. The conference also heard that the Commission and BoMS wanted to avoid fragmentation. This would mean, for example, that instead of having 10 ERNs dealing with sub-sets of neurology, it would be preferable to have an overarching neurology network.

Six steps to ERN status

Stage 1: Call for interest from European Commission

Stage 2: Application submitted to European Commission

Stage 3: Application reviewed by European Commission, then Independent Assessment Body (IAB)

Stage 4: Assessments and reports completed by IAB

Stage 5: Assessment report and recommendations sent to European Commission

Stage 6: Final approval by Board of Member States

A questioner asked whether there would be a mechanism for securing reimbursement if one Member State provides a service to a citizen from another as part of an ERN. In response, it was recalled that the social security regulation and the Cross-border Directive set up the legal framework for the reimbursement procedures which may be relevant. Beside that ERNs could be an opportunity to share expensive infrastructure and diagnostic equipment by instance using structural funds.

Financial sustainability was a recurring theme. The Commission does not have a legal mandate to fund ERNs and thus cannot provide an administration budget. There may be other sources of funding, through the health programme, for example, where funding could be sought. Other avenues, such as seeking structural funds or support from research organisations and the medical industry, could be explored.

Roundtable II: Strategic value of ERNs for the Member States

Chair: Anne Calteux; Ministry of Health Luxembourg

The focus of the presentations was on the benefits that ERNs can bring to European citizens and the role of Member States in bringing the networks to fruition. Opening the session, **Anne Calteux, Ministry of Health, Luxembourg**, pointed to the European Commission report on cross-border healthcare. The document, published in September 2015, showed that patients were not traveling for care as much as had been expected. ERNs could change this, according to the chair, by encouraging patients with rare and complex conditions to seek specialist care beyond the border of the Member State in which they live.

Patrice Dosquet, Ministry of Health, France, said Member States would play a key role in nominating centres of expertise, informing national centres about how to create an ERN, and encouraging centres of excellence to join ERNs. Perhaps the most significant role for national authorities will be the decision to endorse healthcare providers' applications to establish or join ERNs.

France has a well-developed national network of reference centres for rare diseases and is currently working to its second National Plan for Rare Diseases (2011-2016). There are around 380 sites linked to reference centres in academic hospitals. These were designated 10 years ago. "These networks are not responsible for patient care – this is done at a local level – but they share information about complex cases," said Dr Dosquet. "This is still just the beginning; we are just building these national networks."

However, he noted with concern that some well-recognised national experts in France were not involved in developing ERNs and, in some cases, have limited experience of cross-border collaboration. The workload involved in building a network and completing all necessary paperwork could be off-putting, he said. Several other clinicians are very engaged, perhaps because they have previously worked in European projects or pilot reference networks.

Dr Dosquet emphasised that ERNs were about patient care and said they could give patients access to diagnosis for rare diseases. It would, he said, be preferable to develop electronic tools, particularly when it comes to providing remote diagnostic services. "The expertise should travel first, not the patient," he said.

Alexandre Diniz, Ministry of Health Portugal, explained how Portugal has put in place the structures required to support ERNs. The country has integrated the concept of 'reference centres' into its Hospital Network Reform and has started the process of identifying these centres. At present, there are 13 reference centres recognised by the Minister of Health. The Commission for Reference Centres evaluates all the general and specific criteria for the recognition of such centres. These healthcare providers would be well-placed to participate in ERNs, he said.

Portugal's 2015 priority areas for recognition of reference centres are:

- Rare cancers
- Rare diseases (hereditary metabolic diseases)
- Transplantation of solid organs
- Congenital cardiopathies
- Structural intervention cardiology
- Refractory epilepsy

There are now a total of 18 calls for tenders under way in these areas. Healthcare providers in Portugal can apply to these calls, so that they receive national recognition. All of this work brings positives to the Portuguese health system, according to Mr Diniz. "The fact that these centres exist allows us to improve efficiency and effectiveness, improve clinical measures and quality," he said. "From our perspective, one

of the great strategic advances for Portugal is the sharing of knowledge, expertise and experiences.” He also acknowledged the potential of ERNs for achieving economies of scale across Europe and eliminating duplication of effort. The process brings another advantage: it encourages the objective assessment of excellence in healthcare, providing the government with a closer understanding of its hospital network and giving citizens confidence in the system.

Arimantas Tamašauskas, Lithuanian University of Health Sciences, said the development of ERNs was encouraging clinicians, academics and policymakers to focus on patient care. “Despite cultural and national diversity, we can speak the same language on patient-centred platforms where professionals meet to exchange their experience, knowledge and capacities,” he said.

For smaller countries, ERNs are an opportunity to demonstrate their capacity to provide highly-specialised healthcare. Smaller Member States should not be excluded from ERNs because they have lower patient volumes or less sophisticated infrastructure. He said Lithuania has designated four national reference centres in the fields of neurosurgery, oncohaematology, endocrinology and rare heart diseases. All are involved in multidisciplinary work, clinical research, creating guidelines, deploying eHealth solutions and education. These centres are capable of exchanging knowledge and participating in cross-border networks.

Dr Tamašauskas emphasised the need for sustainability given the additional expenses that would be incurred in setting up new administrative structures. He said ERNs opened the door to strengthening the provision of healthcare services for citizens with rare or complex diseases and joint research projects. Lithuanian centres would seek to join ERNs in specific disease areas where there is a strong level of expertise.

Discussion

The key question posed by participants was whether healthcare providers would need to be a national reference centre in order to join an ERN. It was made clear that all healthcare providers would need to be endorsed by their national ministry.

Ministerial high-level roundtable

The conference heard from Health Ministers from Portugal and Luxembourg, both of whom expressed enthusiasm and support for the ERN concept. “I believe we are creating the future of health and differentiated care,” said **Paulo Macedo, Minister of Health, Portugal**. “We must coordinate with the patient in mind.” He also emphasised the need to prioritise patient safety and to share equipment in order to improve diagnostic capacity for severe but rare medical conditions.

Lydia Mutsch, Minister of Health of Luxembourg echoed many of Mr Macedo’s supportive comments. She said most Member States are attracted by the added value ERNs can bring. However, she noted that more needs to be done to raise public awareness of the cross-border healthcare directive, as patients often do not know how to exercise their rights. “Every patient should benefit from the best technology for diagnosis and care. I’m convinced that ERNs will improve access to personalised medicine. ERNs are an opportunity to cultivate a degree of solidarity,” Mrs Mutsch said before stressing the importance of patients’ perspectives and their involvement in the practical implementation of the ERN. In the name of the Presidency, she called on Member States to engage actively with this new experience in order to raise diagnosis and care to the best European standards, but also to ensure the long-term sustainability of centres of expertise and reference networks.

‘WE ARE CONVINCED THAT EUROPEAN REFERENCE NETWORKS WILL DELIVER CONTINUOUS IMPROVEMENT IN THE CARE PROVIDED TO CITIZENS’

Paulo Macedo, Minister of Health, Portugal

Xavier Prats Monné, Director General of DG SANTE, said that the nature of knowledge has changed – it is not merely the transmission of information. Knowledge represents the sharing of information in collaborative networks in conditions of increasing complexity. An ERN represents a network of institutions and people working in a complex and increasingly unstable environment.

Mr Prats Monné said that through ERNs, we will improve “access, effectiveness and resilience of our health systems.” He acknowledged that there are legitimate concerns about financial sustainability, noting that the priority should be to improve services to patients and to share knowledge. IT tools, which should be interoperable, could offer real added value to patients and clinicians alike.

Mr Prats Monné said the ERN slogan – Share. Care. Cure. – was a very good definition of what the networks were all about. At a moment in European history when there are many sources of pessimism, ERNs should give citizens a reason to be optimistic, he said.

Roundtable III: Network organisational challenges and experiences: issues, solutions and lessons learned

Peter O'Donnell; Healthcare Journalist at POLITICO

This session heard from actors with first-hand experience of working in European networks, highlighting the lessons learned and providing tips to those who intend to answer the call for ERNs.

Kate Bushby, EUCERD Joint Action, said a European, holistic approach to caring for rare diseases offers huge opportunities. People with rare diseases currently struggle to access services, while doctors practising in this area have difficulty monitoring the natural history of rare conditions due to the low numbers of patients they work with. EU-funded projects have built up a diverse range of tools and expertise, and ERNs offer a chance to build on this. Looking ahead, there is scope for great efficiency.

In the past there has been a lot of duplication of effort, in that each research project would develop its own website for the duration of its finite funding period. ERNs will be long-term entities. “The big challenge is sustainability,” she said. “Money is an issue that must be addressed at some point. Without a way of sustaining ERNs, there will not be much incentive to get involved.” She said some “glue money” would be required to foster collaborations and keep the network going. “With the best will in the world, a full-time doctor or researcher will not have the time. Without sufficient resources, I can see networks failing.”

Dr Bushby said many current networks are based on research projects and these need to shift focus to patient care if they are to become ERNs. She said the best approach to ensuring all rare diseases find a ‘home’ in an ERN would be to take a structured approach to grouping ERNs into thematic areas. This could require some reorganisation and merging of networks in order to fit into the grouping envisaged by the Commission Expert Group on Rare Diseases Networks.

Ruth Ladenstein of Expo-R-Net pilot network offered the view of a network coordinator with experience working on a three-year pilot project on paediatric oncology. She made a strong case for Europe-wide action on rare childhood cancers, noting that between 10% and 20% of children die from curable forms of cancer where quality care is not easily accessible.

Expo-R-Net has more than 60 partners in 17 countries and involves patients and parents in its governance. It gives children access to specialist advice not available locally by harnessing telemedicine and IT tools. The network links pre-existing reference centres with tumour boards to provide cross-border advice. “Virtual tumour boards are a huge opportunity to enhance cooperation for the future,” she said.

Some tumour types are so rare that there is not yet a standard treatment for doctors to recommend. Building this knowledge database to inform future best practice in treatment is among the future goals of the network.

Dr Ladenstein noted that some countries have low health expenditures rates and have yet to develop national reference sites. Strong e-health tools, provided they are interoperable, can help to raise standards across Europe.

However, she stressed that a cross-border financial contribution would be needed to make virtual tumour boards sustainable. “We are less and less able to spend time on patients that are not our own.”

Yann Le Cam, EURORDIS, offered the patient perspective on ERNs for rare diseases. He said networks are legally obliged to demonstrate ‘patient centricity’, yet the conference room was full of clinicians. While the enthusiasm of healthcare experts is welcome, greater effort will be required to integrate patient voices.

Patients must be the priority of ERNs, Mr Le Cam said, and networks should measurably improve outcomes. He called for greater integration and interoperability of existing systems rather than creating new structures from scratch.

Diseases should be grouped into 'families' which can be addressed by a comprehensive rare disease ERN. While this can be difficult for some smaller disease groups that might prefer to create a large number of highly-specialist networks, the most efficient option is to have between 20 and 25 broad networks. Rare disease ERNs should be 'operational networks', each composed of several 'clinical networks', he suggested. The first step could be to build on the capacities of existing or well-advanced disease-specific networks and registries, and then expand this. "In the long term, each rare disease ERN would ensure that every rare disease patient finds a 'home' in the thematic grouping for his/her disease," he added.

EURORDIS ambition: implementation of ERN to improve patient health outcomes

- Defining patient healthcare pathways
- Identifying the experts and the existing expertise in rare diseases
- Connecting scarce expertise and 'levelling up' knowledge
- Creating the critical mass of rare disease data
- Pushing the pace of rare disease research and daily care practice
- Fostering translational research and therapeutic products into the market
- Engaging patient advocates as actors in healthcare

Discussion

During a Q&A session, speakers were asked whether it would be appropriate to use public money to support the development of orphan drugs which would then be very expensive. Kate Bushby said that where taxpayers had contributed to the development of new products, this should be reflected in the price.

There were warnings that conflicts of interest could arise if ERNs have a role in clinical trials, in providing experts to the European Medicines Agency and in advising HTA agencies. Measures should be taken to prevent such conflicts, it was agreed. The importance of involving experts and patients with knowledge of rare diseases in research and drug development was repeatedly stressed. However, the limited number of patient advocates for some diseases could pose challenges.

Roundtable IV: ERNs and better health outcomes – good clinical practices

Chair: Jorge Penedo; Ministry of Health Portugal

ERNs have the potential to raise standards of care across Europe and, for some rare diseases in particular, could develop guidelines and clinical pathways that do not currently exist. This session explored various forms of clinical guidance, how they are developed and how they can be implemented to improve outcomes for patients.

Paola Laricchiuta of the Istituto Superiore di Sanità, National Centre for Rare Diseases, Italy, said the definitions of guidelines and care pathways can vary with several terms used interchangeably.

One of the key motivations for developing and sharing guidelines is to reduce variability in the delivery of services, bringing healthcare providers up to a shared high standard. This fits neatly with the ERN concept. Similarly, care pathways – the process of steps which are taken throughout the patient journey – can help to make patient management more proactive.

There is, according to existing research, considerable variation across Europe in the production of guidelines and care pathways. While there has been great progress over time, there is still room for improvement.

Domenica Taruscio, also from the **Istituto Superiore di Sanità, National Centre for Rare Diseases, Italy**, noted that article 12 of the Cross-border Healthcare Directive states that ERNs "offer a high level of

expertise and have the capacity to produce good practice guidelines and to implement outcome measures and quality control". Networks can identify diseases where no guidelines exist and work to fill the gaps.

Dr Taruscio highlighted a range of challenges and opportunities in the production of guidelines for rare and complex diseases. She said few countries have well-established systems in place providing mechanisms for quality assurance and implementation of guidelines. "Many countries still rely on sporadic initiatives while others lack the capacity for evidence-based guidelines development," she added.

She said Member States are at a decisive point. The establishment of ERNs is an opportunity to devote sufficient resources to the production of trustworthy guidelines. A cooperative approach to guidelines production and implementation should be embraced.

Karen Ritchie of Healthcare Improvement Scotland said rare diseases experts can learn lessons from how guidelines are used in common conditions. She said there are a range of barriers to the implementation of guidelines, including a lack of professional awareness, lack of skill and lack of will to change practice. It can also be challenging to implement guidelines when patients are living with several diseases or if there is a lack of support for guideline adoption.

Dr Ritchie emphasised the value of measuring outcomes in order to assess whether healthcare providers are meeting their goals. To do this, succinct indicators can help to understand a health system, compare it with other systems and improve it. She gave examples of indicators such as the proportion of cases reviewed at multidisciplinary team meetings and the proportion of patients who die within 30 days of first treatment for a particular cancer.

Looking at the role ERNs can play, Dr Ritchie said the networks could raise awareness of guidelines, develop indicators, and train health professionals to focus on continuous improvement.

Discussion

The issue of conflicts of interest was raised again during the discussion, with two questioners asking how to limit industry influence on guideline development. The goal should be to put health professionals and patient groups into the driving seat, it was agreed.

There was some concern about whether reducing healthcare inequalities through ERNs was feasible and within the remit of the networks. In response, Dr Ritchie said that by implementing guidelines and reducing differences in practice, inequalities could be tackled.

Another speaker noted that guidelines need to be based on high-quality evidence and systemic reviews. This, he said, was fine for common diseases but the lack of data on rare conditions makes it very difficult.

Other suggestions included taking steps to improve literacy of those who must engage with guidelines: health professionals, patients and their parents.

Roundtable V Network Challenges: eHealth and IT solutions

Chair: Jaroslaw Waligora; DG SANTE

Information technology can accelerate cross-border cooperation, facilitating collaboration between experts and online patient consultations. As ERNs prepare for launch, due attention should be given to building systems that improve communication within networks but also between networks. To make this a reality, interoperability is the keyword.

Dr Piotr Czauderna, SIOPEL and Expo-R-Net member, offered attendees an introduction to the Virtual Consultations System (VCS) used as part of the SIOPEL project. The tool allows the management of complex clinical cases, providing users with access anytime from anywhere. There are now 600 tissue samples collected from prospective randomised clinical trials on patients with rare liver cancers. "In my country we have only 15 cases per year so to have several hundreds of cases is a major advantage," he said. Dr Czauderna said the repository of cases means the system can be used as an e-learning tool.

The VCS system is easily adjustable to larger networks and can be used by several experts from various time zones. As long as they have the internet and a laptop, they can log on and review cases.

The system has four profiles:

- A moderator responsible for approval of cases and forwarding cases to competent experts
- Clinicians who can upload diagnostic images and request advice for colleagues
- Panel leaders who facilitate discussion on four key topics (but this number can be expanded)
- Panellists who are experienced clinicians that review cases

Each case is given a unique ID which allows the moderator to track it through the system. The whole review process takes around a week but there is a facility for expediting cases when necessary. The system has an in-built online image viewer, so there is no need to download the large pathology or radiology images that are on the system. The information fed back into the system by panellists is treated merely as advice: the treating physician is still responsible for the care of their patient.

‘INTEROPERABILITY IS AN ATTITUDE’

Henrique Martins

“We believe the system, which took several years to develop, is a fine solution for dealing with the needs of a rare disease network,” said Dr Czauderna. “It can facilitate access to specialist care without moving patients from centre to centre.”

Henrique Martins, EXPAND project coordinator explained that “the challenge is to avoid making information a hostage to one particular country or hospital or software,” as there are pitfalls when designing IT solutions to serve networks of healthcare providers. He said this mistake had been made repeatedly in the past and must not be repeated.

The aim of EXPAND is to support Member States in preparing for the large-scale deployment of eHealth cross-border services. This has clear implications for the building of ERNs and raised a number of questions. For example, could a patient with two diseases be served by two ERNs? And, if they were, would these two networks be able to communicate with one another about this patient? A unique patient identifier is needed in order to connect all the pieces of data related to an individual patient. Ultimately, it should be possible to link eHealth services with social services and even driving licence data.

He said interoperability is “an attitude” and urged people to think of others when designing and using tools: imagine how someone in another part of Europe would create or use a similar document.

There is some cause for optimism that lessons have been learned from previous IT missteps. All 28 Member States have approved guidelines for e-prescription and the EPSOS pilot project has shown that countries can come together to solve their legal and technical issues in an interoperable way.

Contributors from the floor said there is a huge gap between people who talk about interoperability on the one hand, and real-world applications that “are so nice that people fall in love with them”. The temptation is to embrace a tool that suits your immediate needs at the expense of interoperability.

9th October: Plenary session

How to perform a self-assessment and prepare for a strong application?

Chair: Enrique Terol; DG SANTE

Louise Clement and Paula Greco of the PACE-ERN Consortium opened the plenary session with an overview of the assessment process for groups putting together an ERN application. They took participants through the different steps of the ERN application process, reminded them of the timelines and actors involved and offered some tips for self-assessment. They also presented the Assessment Manual and Toolbox (AMT), which was in preparation at the time of the conference, and showed a few examples of operational criteria related to the various categories (network criteria, general provider criteria and specific criteria).

The six-stage process of building an ERN application is expected to take between six and eight months, depending on the complexity of the material. “One of the first steps a group should take is to designate a Network Coordinator and Healthcare Provider Representative,” explained Paula Greco. “Each Healthcare Provider applicant will need the endorsement of the Member State in which it is based. This is something to be secured early in the process.”

For applicants, the most demanding part of the process will be pulling together all the required evidence and ensuring consistency between healthcare providers in terms of their vision for the ERN. Another key element, in addition to describing the network and the disease or conditions it will work on, is communicating the added value that the ERN brings: what is the network doing that is not being done at present and how will this be of benefit to patients in Europe?

Speakers advised applicants to complete the Network Application first and then circulate to Healthcare Providers in the network. From there, each Healthcare Provider should complete the relevant form and the Coordinator would then review all material to ensure consistency.

The Delegated Decision outlines the criteria that Networks and Healthcare Providers must fulfil. The Operational Criteria, which are based on these legal criteria, are central to the process. For each of the criteria there will be a guideline explaining the evidence required and an explanation of how it will be assessed.

Louise Clement said the assessors would strive to reduce duplication of effort by applicants. “For example, if you have a document that covers two of the criteria, submit it and explain that it answers two sections,” she said. “There is no need to generate two identical documents.” Similarly, healthcare providers may find that information related to a centre’s involvement with an existing group covers some of the general criteria that providers must meet as part of an ERN application. This information can be ‘recycled’ rather than creating new paperwork.

Once the application is submitted, it will be formally reviewed for eligibility by the European Commission and IAB. The following assessment process by the IAB may include a virtual interview with the Network Coordinator and members, an on-site visit and a peer-review process.

Workshop 1: How to prepare a successful proposal to build an ERN

Facilitator: Willy Palm, Health Observatory of Health Systems

The workshop was interactive and practical. In addition to hearing from several experts with experience of building cross-border networks, the group broke up into several sub-groups of between 7 and 10 members to address two issues: the governance model of an ERN and the timelines for preparing an ERN application. The full group then came together to share insights.

Central to the debate was the question how to define the scope of ERNs. Given the strong push for the development of inclusive thematic networks, participants discussed whether they, as one clinician suggested, should start small with a narrow focus and scale up or rather begin with a broad umbrella ERN with several sub-networks? For example, grouping all cancers into one network could lead to an exceptionally diverse and potentially cumbersome organisation. A cancer expert in one of the discussion groups said he could readily think of 12 ERNs focused on cancer alone – each with several sub-groups. “To make it manageable, we ought to focus on a small number of cancers as a proof of concept,” it was suggested.

Barbara Brunmair of **ExPO-r-Net**, a pilot network for rare cancers in children, said her advice was to “pick the low-hanging fruit” by identifying networks that already exist and linking them for an ERN application. “My advice is to start with a core ERN and then enlarge it,” she said. “Make sure the Healthcare Providers you include in the application are really expert centres and fulfil the criteria. You can add more centres later.”

**WE WANT A PATIENT-CENTRIC
APPROACH SO WE MUST INVOLVE
PATIENTS FROM AN EARLY STAGE**

Luca Sangiori, Rare Skeletal Diseases network

One participant, who is part of a network focused on rare diabetes in children, suggested the Commission should offer clear advice as to how to address this concern about the scope of the networks “A lot of the conversation we hear from people who want to build an ERN is how to get around the problem – we need guidance on how to tackle this,” he said.

Joan Luis Vives-Corróns said his experience with the **ENERCA network** on rare and congenital anaemias showed how collaborations can improve diagnosis and care, but he added that some haematological conditions could fit into several disease areas and this could be a challenge.

Philippe Ryvlin of the **E-Pilepsy** pilot Network, said it was important to note that not all healthcare providers will be of the same quality. Indeed, if all centres were equally able to deliver the same level of excellence, there would be no value in putting them together – apart from for research purposes. “The primary aim of the ERNs is to work together to create convergence and harmonisation on the quality of care for people with rare and complex conditions,” he reminded the group. Philippe urged those who are embarking on the ERN application process to focus on the added value their network would generate and on the real impact it would have on medical practice.

Luca Sangiori, coordinating a network on **Rare Skeletal Diseases**, said the critical issue for networks making the transition from a national network to an ERN is learning how to change the way they provide assistance to patients. This, he said, led to a clear conclusion: “We want a patient-centric approach, so we must involve patient associations in the governance of ERNs from an early stage. The question we need to answer is how can our ERN meet patient needs that are unfulfilled by current structures,” he said.

This theme of patient-centricity was stressed by several participants from the top table to the conference room floor. Unlike research networks, ERNs are defined by their focus on patient care and this should be borne in mind when building networks. One contributor from the floor proposed that all ERNs should include paediatric expertise as well as patient associations dedicated to children.

ERN-governance model and ERN application

During the workshop, the group was tasked with developing a Gant chart – a timeline – for building an ERN proposal. Some groups used the workshop to flesh out proposals that were quite well advanced: they had provisionally chosen a coordinator, selected some healthcare providers, previously contacted Member States for their endorsement and mapped out a clear work plan to complete the application in time for spring 2016. Others focused on the more generic challenges that lie ahead for those contemplating answering the first call next year. For example, one group sought to describe the ideal ERN coordinator: someone who has enough time and institutional support to devote to drafting the application; is an opinion leader in at least one area of interest; is politically connected; outward-facing and open to taking on new ideas.

Most participants felt that networks could not be too restrictive in the number of partners to include in the application process. In some cases, excluding certain centres from the network would be counter-productive.

The question of setting outcome measures was of concern to several groups. While reducing morbidity and mortality in the chosen disease area would seem like an obvious target, it was thought that this might be an ambitious goal in the first five years. It was also stressed that broad-scope ERNs – such as cancer networks – could find it very difficult to produce ‘deliverables’ given the enormous diversity in the range of diseases they are tackling. Other measures such as building shared IT tools, developing guidelines and growing the network could be considered as useful outcome measures.

Repeatedly the question of financial compensation for services healthcare provided through the network was raised. Also the need for interoperability (an “attitude” rather than a “requirement”) and platforms for data exchange were emphasised, as well as legal guidance on data protection issues.

Securing Member State endorsement was a concern for some healthcare providers. In countries such as France, national networks have been in place for more than a decade. In others, however, there is no formal recognition of networks or centres of excellence, so there is some catching up to do. As Member State support for each healthcare provider is a prerequisite for ERN status, this is a step that should be taken at an early stage, according to the experts.

One important issue raised by several contributors was the need for visibility on ERNs that are in gestation in order to avoid duplication of effort. Some attendees expressed concern that they might invest their time in building a network, only to find that another group is doing the same work in parallel. It was unclear to participants whether the IAB would propose mergers if applications on a particular theme are received from more than one qualified network.

At present, there is no facility at European level for tracking networks in development. At national level, on the other hand, it may be easier to understand what networks already exist or have been endorsed by a Member State. The national representatives in the Board of Member States would have an important

role to play in that respect. It was also suggested that pan-European patient organisations and scientific societies may be a source of intelligence about emerging networks.

On a related but forward-looking note, one contributor proposed that a 'network-of-networks' be established at EU level where ERNs could share experiences and best practice on practical, IT and governance issues. An annual conference could also facilitate information sharing and help to cross-fertilise experience.

In any case, there is a great deal of work ahead so the repeated advice was to "start now".

Workshop 2: How to develop, use and appraise clinical decision making tools

Facilitator: Suszy Lessof, European Observatory on Health Systems and Policies

This workshop looked at the aspects of clinical practice guidelines and decision-making tools that network and healthcare providers (HCPs) need to know about, in order to prepare a successful ERN (European Reference Networks) application.

The overall objectives were a) a discussion of the key challenges of making an application as well as the scope/objectives of the ERN, and b) ways of ensuring that these are all accounted for within a network's operational considerations.

Specific objectives were to discuss i) how a clinical decision-making tool (many of which are freely available on the web) can be incorporated into an ERN application, ii) how an ERN can add value to developing clinical decision-making guidelines (which can vary by network and country of origin), and iii) ways that an ERN can turn its commitments into action.

Looking at network development, the workshop identified six main issues. Firstly, the need to grouping rare diseases under thematic headings and specifically how particular rare diseases (and their existing rare disease networks) identify a 'home'; how the different foci and cultures interact; and how to identify a coordinator / leadership function (given the diversity of conditions).

A further issue is multi-disciplinary networks and how to incorporate them (and different professional perspectives) under the prescribed thematic headings. The third issue is the use of European-developed clinical guidelines within Member States. This can be a challenge due to the different available treatments within each one. Lastly, anyone developing an ERN must look at establishing a common language and terminology; sustainability and resourcing; and support for dissemination and cross-network learning.

It was explained that a significant proportion of the Delegated Decision's conditions and criteria are focused on the delivery and improvements of care. Network applications must show evidence on how the network will deliver care and drive improvements in care through best practices disseminated across the network.

**ERNS SHOULD DEMONSTRATE
A CULTURE OF LEARNING
AND A FOCUS ON QUALITY OF CARE**

Developing guidelines

Turning to clinical practice guideline development, the group voiced a need for more work and/or support in a range of areas. These include defining an appropriate scale / scope for guidelines as well as determining which topics to address (and in engaging patients in priority setting). It will also be crucial (as with network development) to establish a common language / terminology, and to establish an efficient system for developing and implementing guidelines (e.g. central coordinator acting in collaboration with HCPs or local initiatives working in line with a methodology agreed at network level).

Moreover, those developing guidelines will have to understand how to build on existing ones and reconcile different sets of guidelines, and they must accept that guidelines will be applied in very different settings and in countries with different health system structures, funding and expectations. "When developing guidelines, we must be aware of the need for adaptability, with a 360° vision of what is happening in your clinical area from all multidisciplinary team members and from the perspective of patients," added Matt Johnson.

Participants also concluded that expertise should be mobilised when assessing how to develop guidelines and on specific conditions. Moreover, because clinicians have busy working lives, time should be set aside for developing and updating guidelines while avoiding duplication of efforts. Lastly, guidelines updating

should be made a part of the network culture, in response to new research and experience, and there should be sufficient resources (money, time and coordination) set aside for guidelines development and updating.

Building and sharing evidence

In terms of evidence for guidelines, the workshop highlighted the small patient populations for rare diseases, the volume and quality of evidence available, and the predominance of unpublished data (due to difficulties publishing given small case numbers). There are also challenges around undiagnosed diseases and conditions as well as identifying the threshold for classification of an undiagnosed case.

To tackle these challenges, the workshop highlighted the scope for capturing experience systematically and building up evidence through networks. It is also possible to make use of wiki (consensus) models of data collection and improvement. Finally, existing databases and appraisal tools (*RARE-Bestpractices*) can be harnessed to support guideline development for rare diseases.

Paola Laricchiuta, Istituto Superiore di Sanità (ISS) said ERNs need to refer to standards and to produce trustworthy guidelines and to critically appraise existing guidelines. **Domenica Taruscio**, also at ISS, added: “Anyone considering making an ERN application may want to join an ISS course on how to appraise international clinical guidelines. We can offer you appraisal tools which are widely available and free to use, being funded by the European Commission.”

For data collection, the workshop highlighted the importance of using data (or working out how to use it to support and monitor the implementation of change; capture outcomes to allow review and improvement; and make comparisons that support learning (rather than attempting to manage performance or penalise “under-performance”).

Furthermore, common platforms are needed to allow collaboration and learning across the network. Participants also noted the opportunity and challenges in collecting data to add to the evidence base across an ERN, plus the scope for developing prospective databases and registries. **Karen Ritchie, Healthcare Improvement Scotland** said: “There are several useful tools for quality assessment of guidelines, including NICE and GIN.”

Implementing guidelines

The workshop came to several conclusions on guidelines and their implementation. Notably, the need to address how guidelines tackle what to do and how to do it. Some participants also noted the tension between overarching or general guidelines (on ways of working) and very specific ones (on what to do in terms of clinical decisions in specific circumstances).

It will also be crucial to consider how to apply guidelines at different levels of the health system (sub-national, national, European, global) due to varying levels of devolved accountability, budget and decision making in different Member States. Moreover, there are differences in resources and treatment facilities across EU countries, which make mandating particular practices at a European level inappropriate. Hence the importance of looking at how to transfer or adapt existing (high-quality) guidelines for rare diseases from around the world (US, Japan, etc.), so that duplication is avoided but the European context and specificities are reflected.

The participants also noted other issues linked to guideline implementation. Among them are the patients’ rights to access treatment recommended in an ERN best practice. Where treatment is recommended in guidelines but not available to the patient, this can create tension for patients between their European and Member State right to treatment. How should guidelines be applied to centres outside of an ERN that provide care earlier in the pathway? Or when working up patients for referral and treatment in an HCP in an ERN? And how should guidelines be applied for follow-up care so as to ensure that the care needed post-treatment in a HCP in an ERN is respected and to mitigate clinical risk to patients with appropriate after care? Lastly, it was concluded that guidelines could be supported by developing ‘implementation modules’ that provide information relevant to the local health system and context.

Communication, dissemination and promoting change

Participants noted several of their concerns about communication, such as how to do this more widely as well as effectively on specific conditions within networks (data sharing, case consultations, follow-up of cases). There were also questions about how to communicate with and support better practice by countries, institutions and experts that do not want to (or cannot) join the network. Delegates were also concerned with how they can engage with physicians and patients beyond the inner and outer circles that may come across a particular condition very occasionally, so that they can use guidelines to improve care.

Further concerns raised included the need to build in transparency; whether sharing good practices can achieve change more effectively than attempts to mandate changes; and how to deal with territorial ('club mentality') behaviour and attempts by clinicians or institutions to 'keep what is good' i.e. patients, funding or knowledge for themselves.

As for engaging with patients, there was agreement on the value of involving patients in developing guidelines, as well as setting priorities and the agenda for guideline development. It is also vital to incorporate patients' perspectives in network learning and improvements in care, in line with the goal of patient-centred care. "Guideline development is all about clinicians and patients talking to one another with a focus on decisions to be made, as well as uncertainties as to how a healthcare community can move forward," said Michele Hilton-Boon, University of Glasgow.

Guidance needed

As a result of this workshop, the most clearly and frequently expressed 'needs' were for guidance on which conditions, individual (existing) networks and interested parties fit under the published disease groupings. This is particularly relevant for 'mixed' conditions with multi-disciplinary responses. Support was sought in identifying a coordinator from an HCP for each new network.

Participants stressed the importance of resources – particularly funding but also IT platforms and tools – to allow networks to be sustainable and to support network development and dissemination.

Workshop 3: Clinical Research in a Network Environment

Facilitator: Irene Glinos, European Observatory on Health Systems and Policies

Article 12(4)(a) of Directive 2011/24/EU identifies research as an important priority for ERNs. The Commission Delegated Decision specifies three criteria and conditions to be fulfilled in this area: to identify and fill research gaps; to promote collaborative research within the network; and to reinforce research and epidemiological surveillance, through setting up of shared registries.

Workshop III examined several issues related to the integration of research into ERNs, including possible collaboration with the EC's Joint Research Centre (JRC), clinical data sharing across network partners, hosting of innovative clinical trials, and interactions with industry.

Zuleika Saz-Parkinson (JRC) discussed how the JRC, the EC's in-house science service, could work with ERNs. To support epidemiological studies and policymaking, the JRC is building a comprehensive **European Cancer Information System**, working with multiple partners to establish a framework to ensure the interoperability of national and international cancer registries. Since 2012, it has hosted the secretariat of the European Network of Cancer Registries, whose aims include enhancing collaboration, defining data standards and supporting dissemination of information.

The JRC is also working with DG SANTE to develop an **EU Platform on Rare Disease Registration**, focusing on (1) interoperability of the 600 or so rare disease registries that currently exist in the EU and (2) sustainability of surveillance networks. The central registry and European-level coordinating activities have been transferred to the JRC for EUROCAT (European network for the surveillance of congenital anomalies) and will be soon for SCPE (Surveillance of Cerebral Palsy in Europe network).

In the field of health quality assessment, the JRC is leading the **European Commission Initiative on Breast Cancer (ECIBC)**, which is developing a set of evidence-based requirements to define quality of care and how it is measured. ECIBC's work may cover rare breast cancers, which would be relevant to an ERN focused on this theme.

Pascale Flamant (UNICANCER) described a possible model for sharing health data, the **ConSoRe** (Continuum Soins Recherche) system. The system is designed to provide access to data across 20 French Comprehensive Cancer Centres, which have a strong emphasis on research.

Information is stored locally, in multiple databases (structured and free text). ConSoRe works at a local level, providing integrated access to data across these multiple sources, as well as access at a national level to anonymised summary data. Following a proof of concept study at four centres, the system is being refined for wider rollout in 2017 (although further funding needs to be secured).

As well as technical issues, the project had to address the concerns of physicians about allowing wider access to data as well as legal data privacy issues, discussed with the French data privacy agency. Data sharing across national borders would add further legal complexities, and international sharing of health data to support research may be an issue requiring greater EC attention.

Maurizio Scarpa (Helios Horst Schmidt Kliniken, Wiesbaden, Germany) highlighted the potential role for ERNs in facilitating clinical trials on rare diseases, particularly in children. For multiple reasons, organising trials in young people with rare diseases is challenging, and traditional randomised controlled trials are not well suited to the assessment of new treatments.

ERNs provide a framework in which innovative new trial designs and statistical methodologies could be developed, and natural history studies conducted. In addition, they could have wider influence as a focal point for rare diseases, in dialogue with regulatory agencies, working with patient associations, and establishing new models for interactions with industry.

Denis Lacombe (European Organisation for Research and Treatment of Cancer, EORTC), raised the potential for radical new approaches. He argued strongly that ERNs need to integrate research and clinical care, creating platforms to generate a better understanding of disease, to develop and test new therapies, and to follow up patients to analyse outcomes – thereby accelerating the development, testing and implementation of new therapies.

Through EORTC's SPECTArare initiative, a sophisticated infrastructure has been established by academic partners to characterise cancers. In collaboration with companies, this information is then used to match patients with appropriate clinical trials, while follow-up of patients allows the collection of outcomes data. Liaison with regulators is supporting the use of innovative trial designs, and discussions with payers can ensure that trial and follow-up data feed into regulatory decision-making, promoting rapid access to new treatments. Although primarily a research initiative, SPECTArare fulfils many ERN criteria. However, establishing disease-characterisation platforms requires considerable investment, likely to be beyond the capacity of a single healthcare provider. Furthermore, the complexity of the proposed application procedure may strongly discourage centres from participating in an ERN.

Samantha Parker (Lysogene) discussed how ERNs could be made attractive to industry and a basis for effective collaboration. She suggested that industry and ERN partners shared common objectives – improved patient care – but it was important to acknowledge upfront that the two communities had different primary aims. Open and honest interactions would be key to successful collaborations. ERNs could enable healthcare providers and industry to work together to improve processes. One priority could be greater standardisation – methods used to assess patients vary widely, making comparisons difficult. Assessment of natural histories could also be coordinated better, so families are assessed once as part of different studies. ERNs could also provide opportunities to collect post-marketing safety surveillance data, to support expedited licensing of new agents. While ERNs are likely to be attractive to industry, sustainability will be an important issue, if industry is to invest time and resources in establishing collaborations. There could be potential for cost-sharing agreements, and it would be helpful if ERNs were established as discrete legal entities.

As raised by several participants in discussions, industry involvement would need to be managed to avoid conflicts of interest in areas such as guideline development and training. Establishing clarity on roles and responsibilities would be of paramount importance.

Workshop 4: Rare Disease Networks - lessons learned, grouping diseases and patient involvement

Facilitator: Matthias Wismar, European Observatory on Health Systems and Policies

Attendees considered how best to establish an ERN and the challenges involved. **Yann Le Cam**, a co-founder of the EURORDIS organisation, which campaigns on behalf of patients with rare diseases, outlined the need to involve patients both in the application process and the ongoing running of ERNs. He said it was important to have at least one patient representative on an ERN board.

“There remains a need to continue to raise awareness of the proposed approach,” Mr Le Cam said. “Patients should be allowed to participate in the ERN board meetings to ensure discussions are patient-centred, and to contribute to the development, application and evaluation of ERNs. This will ensure all patient representatives for all disease groups are covered.”

He added that ERNs should use patients in the development of standards and guidelines, so as to align clinical and patient needs. ERNs should also consider the expectations and experience of patients in ‘strategic work’, which includes health and social care, public health and human rights.

Professor Kate Bushby, a clinical academic professor across Newcastle University and Newcastle Upon Tyne NHS Foundation Trust, emphasised the value of people who can coordinate complex multidisciplinary structures: “We need experts in running networks as much as experts in disease areas.”

Developing an ERN proposal

Delegates in five groups imagined they were developing a proposal to become an ERN and considered how to go about this. They highlighted the extra benefits to existing networks and centres specialising in specific rare diseases of working together in broader thematic networks. They also analysed how the specificity of individual rare diseases could be maintained within the context of thematic networks.

Some delegates said there could be a challenge to create an atmosphere of co-operation between clinicians who are in some senses in competition. **Victoria Hedley**, an assistant manager at the **EUCERD Joint Action: Working for Rare Diseases** project, who is based in Newcastle,

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Professor Kate Bushby

UK, said: “We want collaboration not competition. Research is competitive by its very nature. We want to enhance the profile of the centre, so we have to get that collaborative support.” She added that ERNs cannot exist in isolation. “There must be contact across networks, especially to help undiagnosed patients.”

The importance of sharing patient databases and registries was frequently highlighted. Funding was an issue that concerned some delegates. One said: “Money is a key thing. If I go to my boss and I say I want to apply to run an ERN, he will say: ‘Will it bring in extra money for our hospitals? If it won’t he will just say ‘no’, because he will think there is no point in just have a logo saying we have an ERN.”

Another contributor argued that applications for ERN status should emphasise the opportunities for enhancing patient care. It was noted that the added value of ERNs is that they should bring better service provision, better clinical and laboratory work, more research and better education.

A clinician argued that ERNs will also empower patients. “If you have registries for use across the EU, it will bring added value for service provision. But there is some ambiguity about where conditions including neuro-muscular diseases would sit among the 21 categories.”

ERNs would provide added value in the sense that they will have prestige, which could have financial benefits, said a delegate representing one discussion group. Accredited ERNs may find it easier to attract research grants, it was suggested.

How to empower patients

In this section, delegates considered how patients could be involved and empowered. What would their role be, and how would ERNs ensure that their model was patient-centred?

One delegate said their network would have surveys, focus groups and meetings to engage patients. Other participants with experience in this area agreed that this is a useful approach.

One of the discussion groups said patients should be invited to draft their own bill of rights as part of ERN activities. A participant said patients would need support if they are to inform the development of best practice guidelines: "You won't be able to just tell patients to prepare best practice guidelines; you will need to give them support."

A discussion group spokesperson said that a way of involving patients in research is to ask them to list ten priorities they would like to see for research projects. A separate group concluded that patients should have a representative on the ERN board. "We need leaflets explaining how the network centres work within the community and to have patient days where we explain to them how it works."

It was emphasised by one participant that patients need representation within the ERN; they would also value "self-management support, continuous care and access to second opinions." In response, another delegate said many patients will not want involvement in the ERNs, so it will be down to doctors to represent their views.

Conclusions from workshop groups: recurring themes, points and challenges

- ERNs must seize the chance to co-operate by sharing patient data and registries.
- ERNs will have a challenge in deciding where to place undiagnosed patients, but there are potential benefits in improving care and achieving diagnoses for these patients.
- Patients must be involved in setting standards and creating best practice guidance.
- Patients should have representatives on ERN boards.
- ERN co-ordinators should be given funding and/or admin help for their roles.
- Setting up an ERN in the cancer field is challenging, partly because it is such a wide area; representing all types of cancer will be difficult.
- ERNs need a single IT platform to share ideas, records and best practice electronically.