EUROPEAN COMMISSION
HEALTH AND CONSUMERS DIRECTORATE-GENERAL
Health systems and products
Healthcare Systems (D2)

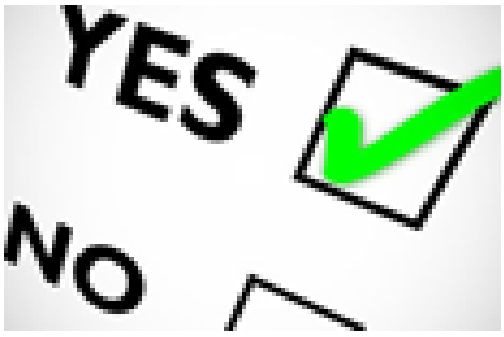
Brussels, June 2013

Summary report of the replies on the

PUBLIC CONSULTATION

ON THE IMPLEMENTATION OF EUROPEAN REFERENCE NETWORKS (ERN)

Directive 2011/24/EU of the European Parliament and of the Council of 9 March 2011 on the application of patients' rights in cross-border healthcare



Disclaimer:

This paper should be regarded solely as a summary of the contributions made by stakeholders to DG Health and Consumers' public consultation on the implementation of European Reference Networks (ERN) under the framework of the Directive 2011/24/EU of the European Parliament and of the Council of 9 March 2011 on the application of patients' rights in cross-border healthcare. It cannot in any circumstances be regarded as the official position of the Commission and its services

Contents

1. INTRODUCTION

Directive 2011/24/EU of the European Parliament and of the Council of 9 March 2011 on the application of patients' rights in cross-border healthcare requires the European Commission to support Member States in the development of European Reference Networks composed of healthcare providers and Centres of Expertise.

The main added value of the European Reference Networks and of the Centres of Expertise is the improvement of access to both diagnosis and high-quality, accessible and cost-effective healthcare for patients who have a medical condition requiring a particular concentration of expertise or resources, particularly in medical domains where expertise is rare. European Reference Networks could also be focal points for medical training and research, information dissemination and evaluation, especially for rare diseases.

Article 12 of the Directive requires the Commission to adopt a list of criteria that the networks must fulfil, and the conditions and criteria which providers wishing to join networks must fulfil. The Commission is also required to develop and publish criteria for establishing and evaluating European Reference Networks. And it must facilitate the exchange of information and expertise on the establishment of the networks and of their evaluation.

DG Health & Consumers launched a public consultation targeted to stakeholders, on the European Reference Networks (ERN) and in particular on the criteria to be considered according to Article 12 of Directive 2011/24/EU.

The objective of the consultation was to seek the views of interested parties on the potential scope of European Reference Networks, and the criteria for healthcare providers wishing to join them. In particular, it sought opinions and contributions based on evaluated experiences, regional or national models, technical and professional standards, criteria or recommendations which could facilitate the definition of technical and quality criteria (scope, general and disease specific elements).

This consultation consisted of an online survey which was open for submission for 12 weeks (23 November 2012 - 22 February 2013).

This document summarises the contributions made by stakeholders on the elements to be addressed in the implementation of Article 12 of the Directive 2011/24/EU on European Reference Networks (ERN), and in particular on the criteria to be considered in the process of identification and designation of healthcare providers as Centres of Expertise.

This summary report gives an overview of the responses and describes the main outcomes of the consultation. It is divided in four main sections:

1.- The profiles of the respondents

2.- The involvement of the respondents in the field of highly specialised healthcare

3.- The opinion of the respondents on the proposed criteria for ERN (scope, general and specific criteria)e document

4.- The annexes (4) including detailed information on the respondents , the criteria, the textual comments and the general statistics of the public consultation.

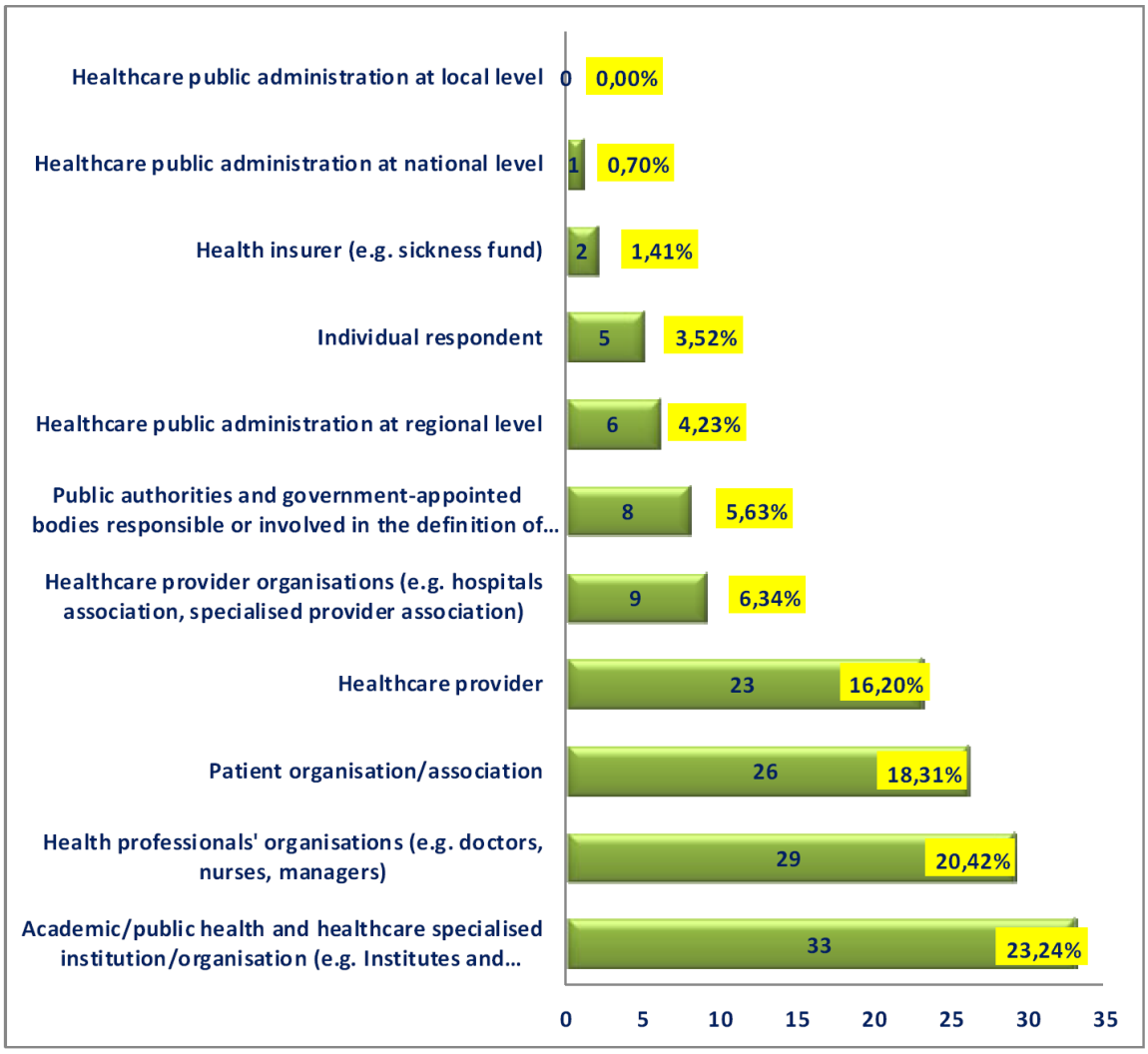
An exhaustive repository of all the individual responses is accessible in SANCO's website:
http://ec.europa.eu/health/cross_border_care/consultations/cons_implementation_ern_en.htm

2. PROFILE OF THE RESPONDENTS

The Commission received **138 contributions**. The respondents identified themselves from a list of 10 possible options (which covered organisations, institutions and individual respondents). **Figure 1** illustrates the distribution of respondents. Four types of organisations represented almost 80% of the respondents.

Regarding the territorial scope of the organisation of the respondents, 28 contributions (20,29%) were received from EU-wide organisations. 21 (75%) of these define themselves as pan-European; 7 (25%) cover between 10 and 20 Member States (more detailed information is in annex 4.1)

Figure 1: Distribution of responses according to type of organisation

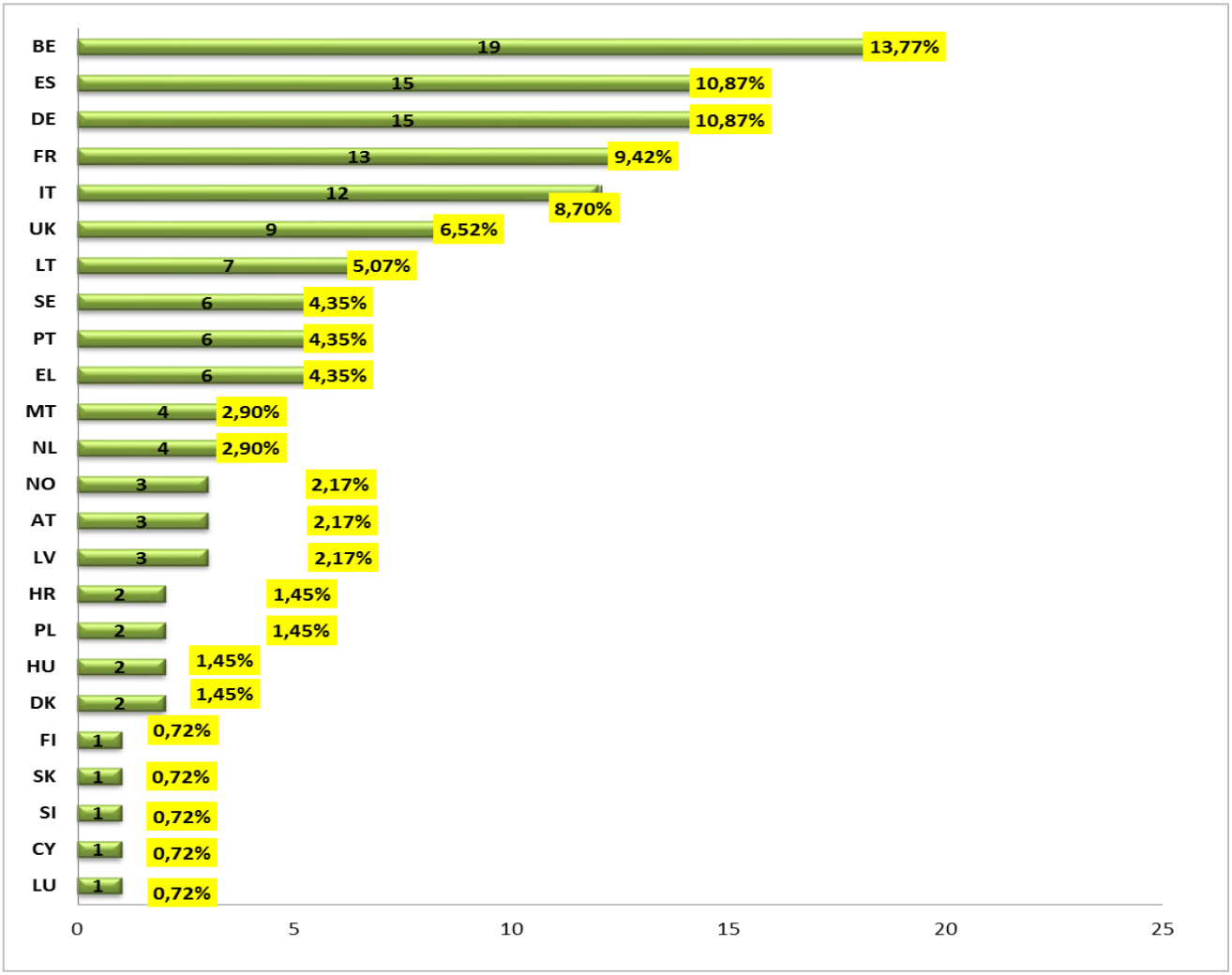


Furthermore, 13 (9,42%) national and regional authorities also responded to the stakeholder consultation.

133 (96,37%) responses came from organisations and 5 (3,52%) responses from individual citizens. The main contributions from organisations were received from academic/public health and healthcare specialised institutions/organisations 33 (23,91%) and from health professionals' organisations 29 (21,01%).

Figure 2 shows the distributions of responses across the different EU Member States and EEA countries (138 in total, 100 % of replies), with the largest groups contributing being from Belgium, Germany, Spain, France and Italy. There were no replies from 5 EU Member States. Norway was the only EEA country contributing. No replies were registered from outside the EU.

Figure 2: Country distribution of responses



A majority of respondents (73, or 52,90%) conduct business or are represented in only one EU Member State/EEA country. 32 (23,16%) of the contributions came from respondents covering between 2 and 14 EU Member States/EEA countries. 33 (23,9%) came from respondents covering between 15 and 31 EU Member States/EEA countries.

Almost all the respondents (134, or 97,10%) agreed to be contacted after the consultation. A list detailing all contributors is provided in the Annex 1 to this document. All the comments and replies are detailed in Annex 3 and 4.

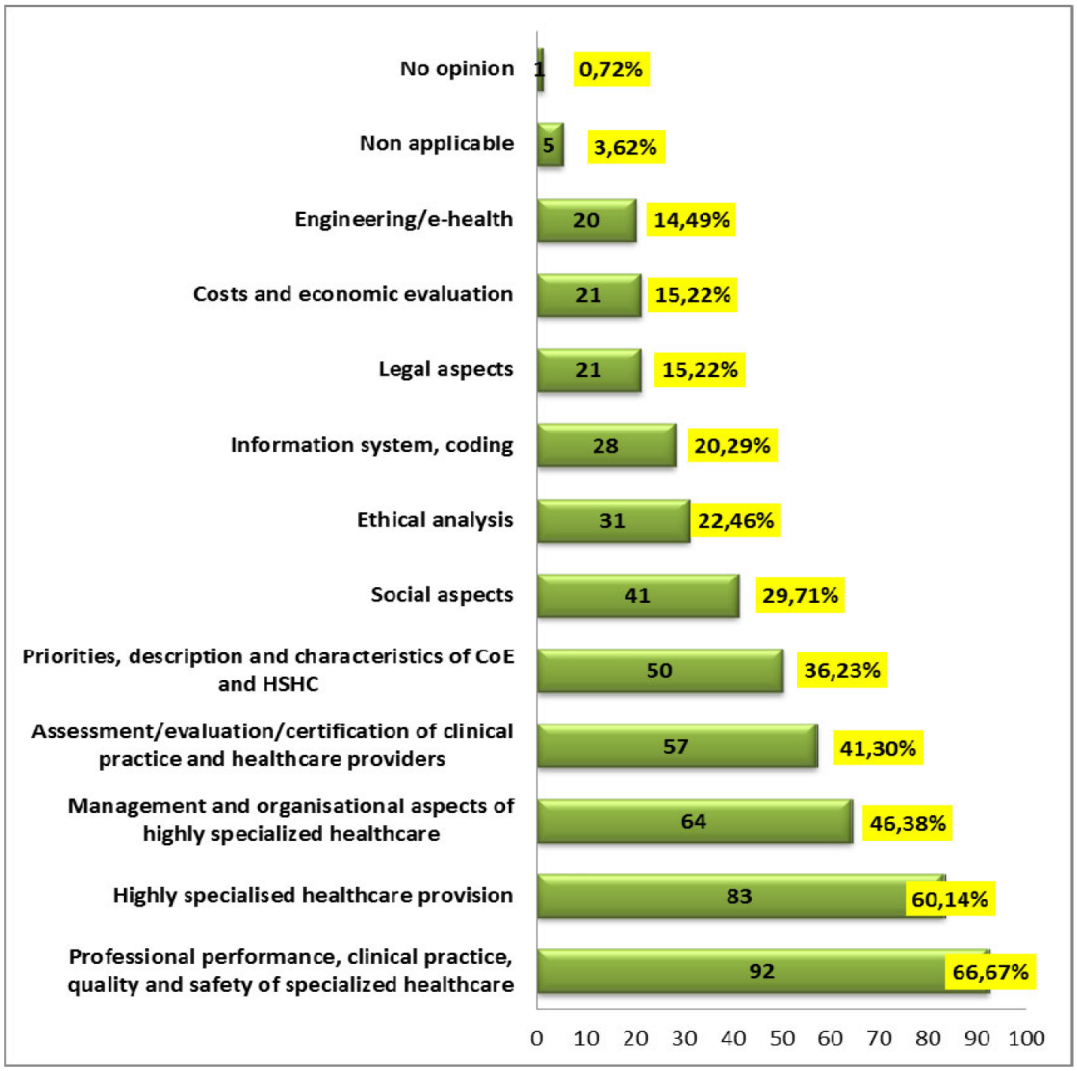
The responses provided by stakeholders have been published on the "SANCO website" of the European Commission.

3. Involvement of the organisation in the matter of centres of excellence/reference (COE) and healthcare networks in highly specialised healthcare (HSHC)

100 (72,46%) respondents stated they had either 'very good' or 'good' knowledge of the subject matter.

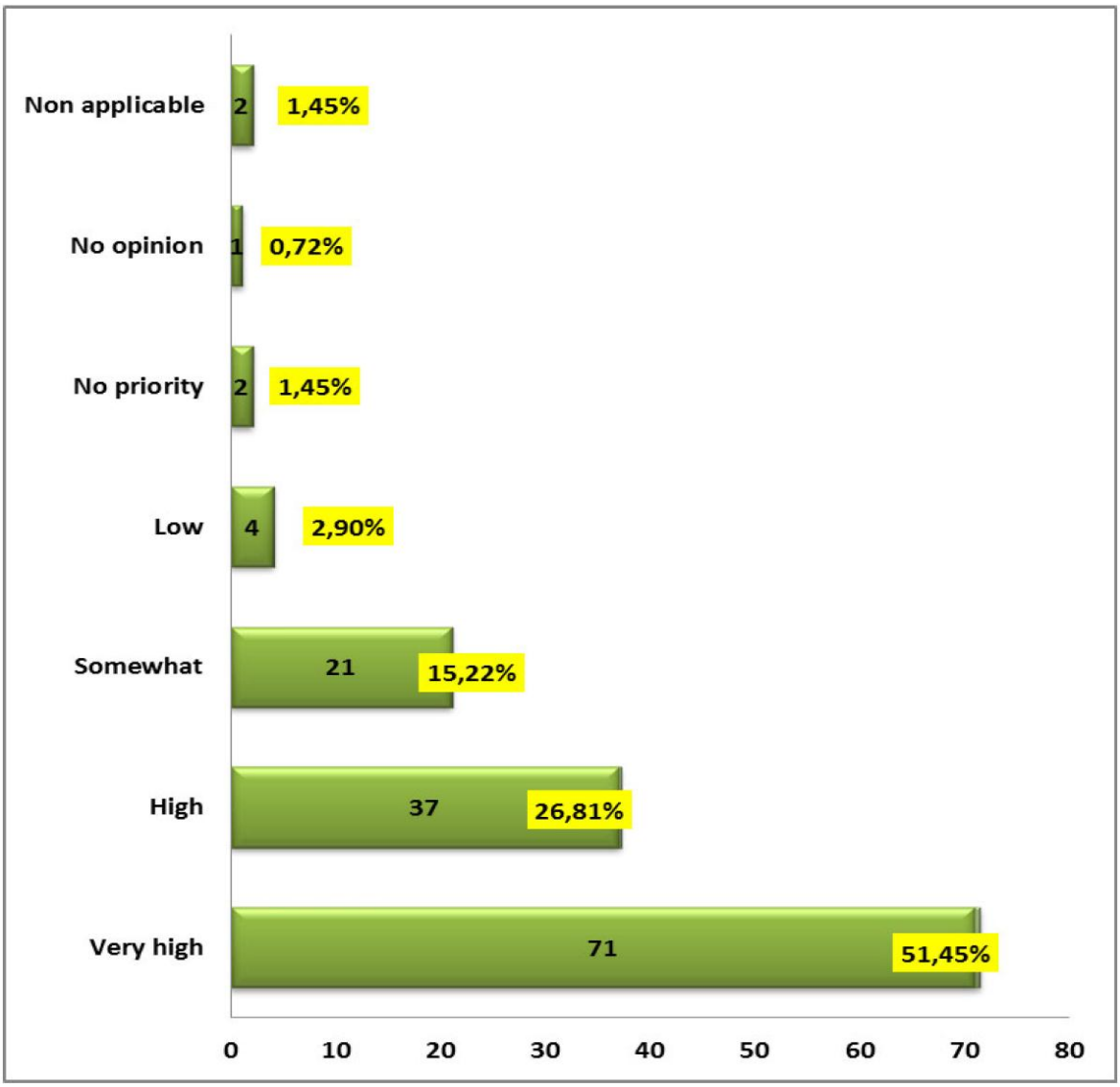
Figure 3 shows the subjects or fields in which organisations considered themselves to have specialist knowledge as regards centres of expertise, networks and highly specialised healthcare. The main three areas of knowledge are 'professional performance, clinical practice, quality and safety of specialised healthcare', 'highly specialised healthcare provision' and 'management and organisations aspects of highly specialised healthcare'.

Figure 3: I Organisation's key knowledge in domains related to the topic of centres of expertise, networks and highly specialised healthcare (multiple choices)



Among the total number of respondents, 108 (78,26%) stated that highly specialised healthcare was a 'very high' (51,45%) or 'high' (26,81%) priority in their organisation's strategies and work plans (See **Figure 4**).

Figure 4: Ranking of highly specialised healthcare as a priority in the organisation's strategies and work plans

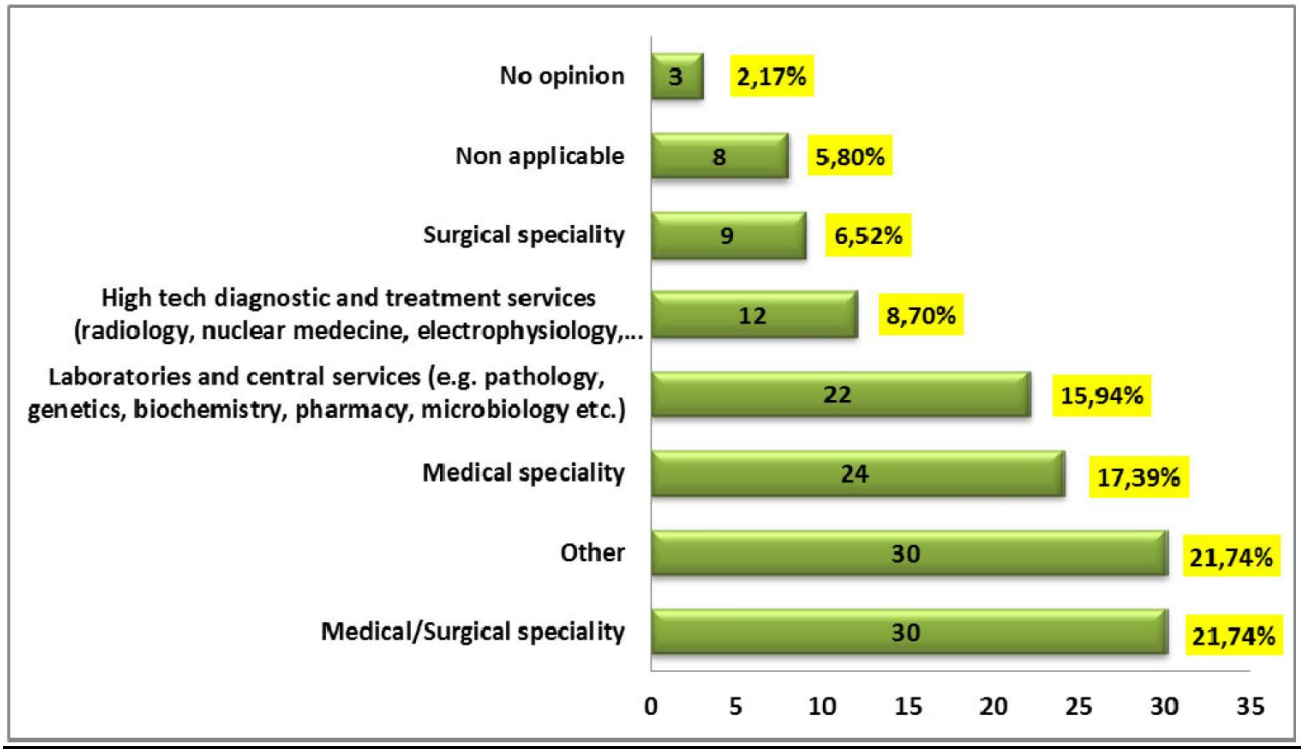


In the specific field of healthcare services/specialities which are the most relevant for the centre/organisation's field work, **Figure 5** shows that the main categories mentioned are:

- Medical/surgical and surgical speciality
- Medical speciality
- Central services (laboratories and central services and high tech diagnostic and treatment services).

Figure 5: Classification of the specific field of healthcare services/specialities in terms of relevance for the centre/organisation's field of work

Most relevant specific field of healthcare services in your organisation



As shown in **Figure 6,** a majority of respondents have been involved in the design or assessment of professional standards and criteria related with highly specialised healthcare.

Figure 6: Involvement of the organisation/centre in the design or assessment of professional standards and criteria related with highly specialised healthcare

	Number	% of (138)
Frequently	59	42,75%
Occasionally	50	36,23%
Never	21	15,22%
Non applicable	4	2,90%
No opinion	4	2,90%

Of the 138 respondents, 46 (33,33%) stated that their organisation/centre has been involved in projects/activities supported by the Commission in relation with healthcare networks in highly

specialised healthcare (HSHC) or professional and technical criteria/standards for highly specialised healthcare. In addition, based on their own organisation's experience, 90 respondents (65,22%) were able to provide references or links to documents related with professional criteria and standards for Centres of Expertise (CoE) or highly specialised healthcare (e.g. quality criteria, guidelines, consensus documents).

Of the 23 respondents who identified themselves as healthcare providers, 20 (86,96%) indicated that their centre or unit is directly involved in the management (diagnosis, treatment, etc.) of highly specialised diseases or conditions. Of these, 17 (73,91%) are part of a centre or unit designated or recognised as centre of reference/excellence in their country.

Concerning the process of designation/recognition as centre of reference/excellence, 7 (41,18%) indicated a formal institutional process, 7 (41,18%) indicated a designation/recognition based on professional recognition, and 3 (17,65%) indicated 'other'.

Of the 17 respondents designated as centre of reference/excellence, 15 (88,24%) are currently participating in a network of centres of expertise. For 9 (60%) of them, the scope of the network is international. Otherwise, the scope of the network remains national for 5 (33,33%) of them and regional for 1 (6,67%) of them.

When asked about the source of support for the network, 6 (40%) indicated it was national, 6 (40%) indicated it was international, 2 (13,33%) are funded by other sources and 1 (6,67%) is funded by the European Commission (**see Figure 7**).

Figure 7: The kind of the network and support

	Number	% of (15)	% of (138)
National	6	40,00%	4,35%
International	6	40,00%	4,35%
Funded by other sources	2	13,33%	1,45%
Funded by the European Commission	1	6,67%	0,72%

Furthermore, as shown in **Figure 8**, almost all the providers (18 of 20 respondents, or 90%) with experience in the process of management (diagnosis, treatment, etc.) of highly specialised diseases or conditions expressed their interest in applying to be considered as a Centre of Excellence within a future European Reference Network.

Figure 8: Degree of interest in applying to the process to be considered as a Centre of Excellence of a future European Reference Network (*1 = not interested at all, 5 = very interested*)

Level of interest	Number	% of (20)	% of (138)
5	16	80,00%	11,59%
4	2	10,00%	1,45%
2	1	5,00%	0,72%
3	1	5,00%	0,72%
1	0	0,00%	0,00%

3. SUMMARY OF THE RESULTS AND CONTRIBUTIONS

A list of the proposed criteria for ERNs (scope, general and specific criteria) is provided in Annex 3.

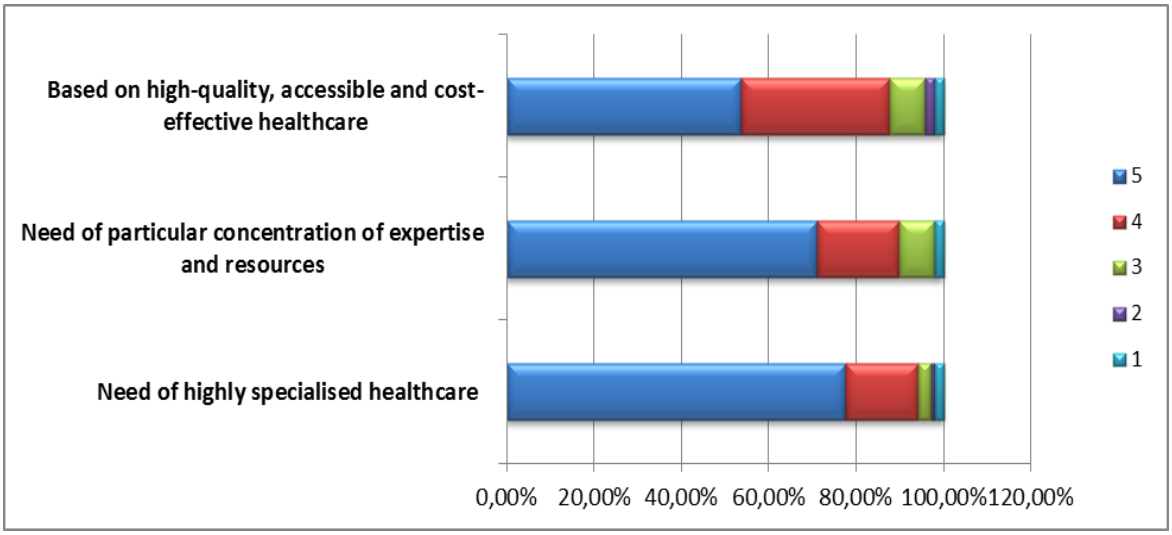
a.1. Views of respondents on the criteria related with diseases or conditions in order to be considered under the scope of the ERN

As a first step, respondents were asked to provide their view on the relevance of possible criteria that the condition, disease or technique should fulfil to be considered for inclusion in the scope of the future ERN. They were asked to rate from 1 (not important) to 5 (very important) the importance of each criterion.

The overall assessment shows that respondents considered all proposed criteria relevant: on average 125 respondents (90,58%) saying each was 'very important' or 'important'. The criterion "need of highly specialised healthcare" received the most support with 130 respondents (94,21%) saying it was either 'very important' or 'important'.

Figure 9 illustrates the ranking of the above mentioned criteria according to the responses.

Figure 9: Relevance ranking of the criteria proposed (*1 = not interested at all, 5 = very interested*)



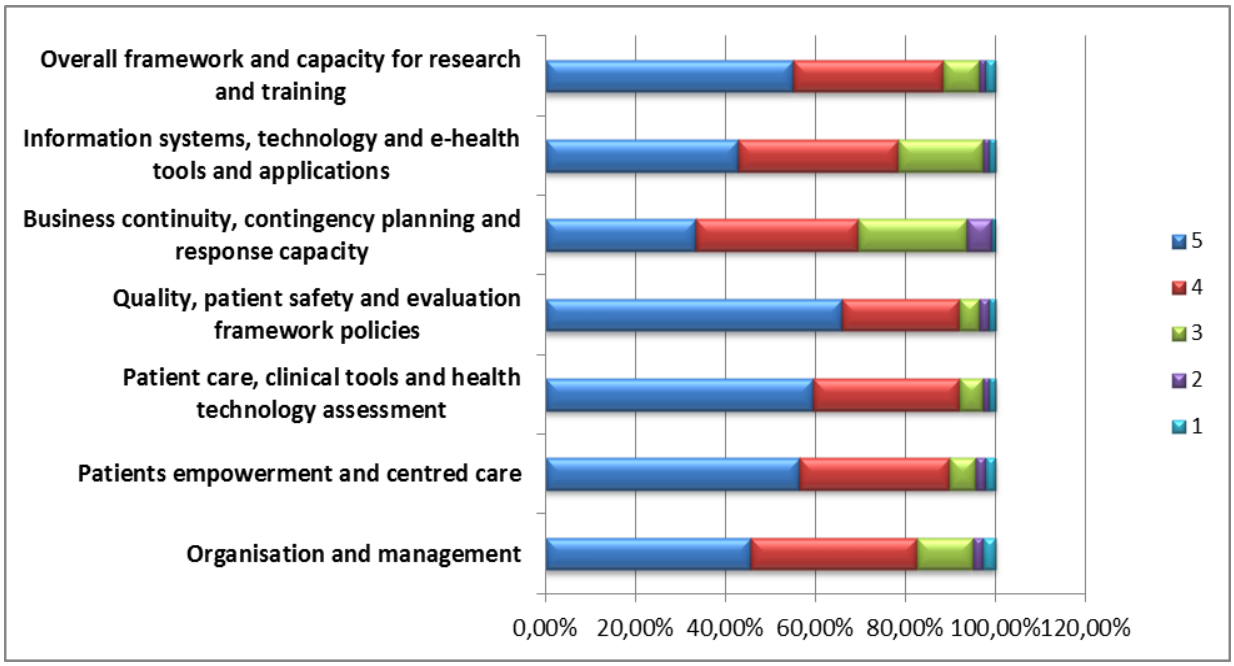
A majority (89, or 64,49%) of respondents thought that there was no need for additional criteria or option to address the issue. 88 (63,77%)respondents noted that the diseases or group of diseases to be addressed by the future ERN would need to be prioritised according to the proposed criteria.

a.2. Views of respondents on the general criteria of the centres wishing to join a European Reference Network

To help identify and define the criteria to be used for the assessment and designation as centre of reference of any type of healthcare provider, respondents were asked to rate the importance (value and pertinence) of the proposed criteria and sub-criteria from 1 (not important) to 5 (very important).

All the general criteria proposed were considered to be relevant by a large majority of respondents (on average 117 respondents or 85,14% either 'very important' or 'important'). The strongest support (>92%) was for the criteria ‘quality, patient safety and evaluation framework policies’ (127 respondents or 92,03% 'very important' or 'important') and to ‘patient care, clinical tools and health technology assessment (127 respondents, or 92,03% 'very important' or 'important').

Figure 10: Relevance ranking of the proposed general criteria (1 = not interested at all, 5 = very interested)



A majority of 71,74% (99) of the respondents agreed that there was no need for additional option to address the issue.

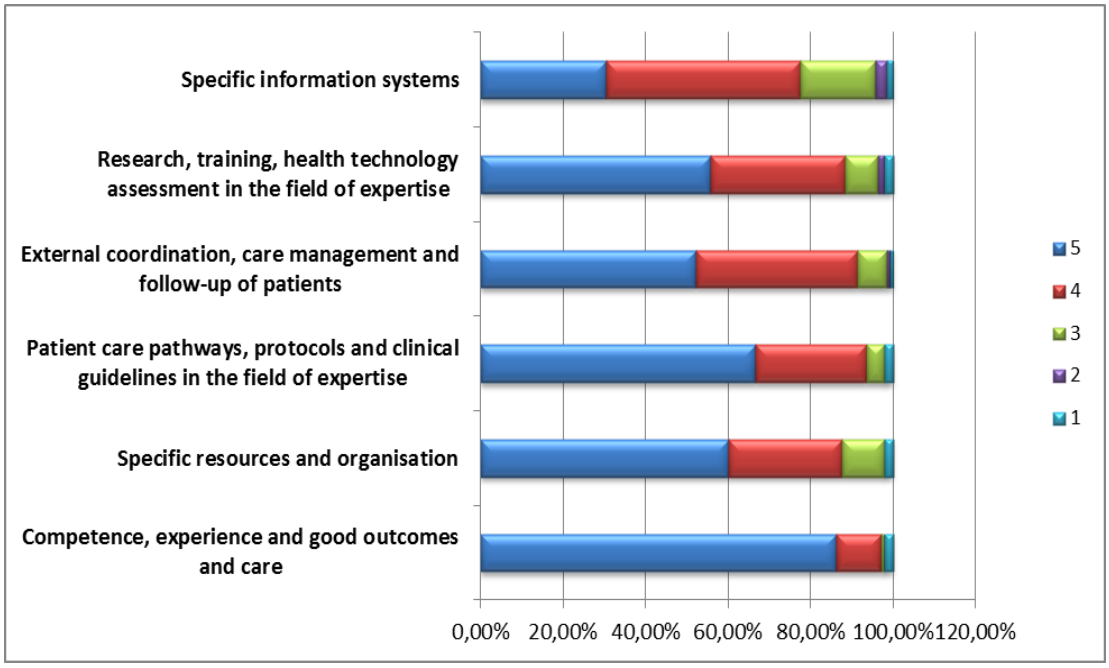
a.3. Views of respondents on the specific criteria regarding the areas of expertise

Stakeholders were invited to provide their views on the specific fields and elements which could be used for the assessment of a healthcare provider wishing to be selected as a centre of reference for a specific disease or condition. They were asked to rate the importance (value and pertinence) of the elements to be addressed by the proposal criteria and sub-criteria from 1 (not important) to 5 (very important).

A majority of respondents considered all the proposed criteria to be relevant (an average of 123 respondents or 89,24% saying each was either 'very important' or 'important'). The strongest support (> 90% very important and important) was for the following criteria:

- Competence, experience and good outcomes and care (134, or 97,1% 'very important' or 'important')
- Patient care pathways, protocols and clinical guidelines in the field of expertise (129, or 93,48% 'very important' or 'important')
- External coordination, care management and follow-up of patients (126, or 91,3% 'very important' or 'important').

Figure 11: Relevance ranking of the proposed specific criteria (1 = not interested at all, 5 = very interested)



108 respondents (78,26%) did not think any additional criteria or option was needed.

ANNEXES

Annex 1: List of contributors to the public consultation

138 contributions were received. Contributors are listed in alphabetical order for each of the eight possible categories maintaining the self-classification made by them. Some of the contributors could have been classified under different categories.

1. ***Academic/public health and healthcare specialised institution/organisation (33)***

- ✓ *Attikon Hospital, Greece*
- ✓ *Berufsgenossenschaftliches Unfallkrankenhaus Hamburg, BG Trauma Hospital Hamburg BG= German Social Accident Insurance Institution for trade and industry),Hamburg, Germany*
- ✓ *Centre de Référence des infections ostéo-articulaires complexes pour l'Inter-région Nord-Ouest, France*
- ✓ *Children´s Heart Center, Skane University Hospital, Lund, Sweden*
- ✓ *Clinica Pediatrica Università degli Studi dell'Insubria - Ospedale di Circolo Fondazione Macchi - Varese – Italy*
- ✓ *Clinical Neurosciences Department, Neurology Unit, Italy*
- ✓ *Coordinating Centre for Rare Pediatric Diseases, Lithuania*
- ✓ *Croatian National Institute of Public Health. Croatia*
- ✓ *Department of Clinical Sciences, Lund University, Plastic and Reconstructive Surgery, Jan Waldenströms gata 18, Skåne University Hopsital, SE-20502 Malmö, Sweden*
- ✓ *Department of Neurosurgery, Lithuanian University of Health Sciences and Hospital of Lithuanian University of Health Sciences; Neuroscience Institute, Lithuanian University of Health Sciences. Lithuania*
- ✓ *DNV Business Assurance, Norway*
- ✓ *Erasmus MC, Center for lysosomal and metabolic diseases, Netherlands*
- ✓ *European network of paediatric research at the European Medicines Agency (Enpr-EMA)*
- ✓ *European Union of Medical Specialists, Belgium*
- ✓ *Fondation René Touraine: an international Foundation for Dermatology*
- ✓ *French Reference Center for Rare Iron Overload Diseases of Genetic Origin. France*
- ✓ *German Mesothelioma Register. Germany*
- ✓ *Hospital La Fe, Spain*

- ✓ *Institute of Child Health, Greece*
- ✓ *IRCCS - Instituto Ortopedico Rizzoli - Department of Medical Genetics and Rare Orthopaedic Diseases, Italy*
- ✓ *Istituto Ortopedico Rizoli, Italy*
- ✓ *National Center for Tumor Diseases (NCT) Heidelberg.Germany*
- ✓ *Neuromuscular Disorders Clinic, Neurology Department, Hospital General Universitari Vall d'Hebron, Spain*
- ✓ *North of England EU Health Partnership. United Kingdom*
- ✓ *RCCRD, University Hospital of Udine, Italy*
- ✓ *Red cell pathology unit. Hospital clinic-university of Barcelona, Spain*
- ✓ *Regional Coordinator Centre for Rare Diseases of the Friuli Venezia Giulia Region-Italy*
- ✓ *Riga East University hospital stationary "Infectology Center of Latvia" (RAKUS LIC). Latvia*
- ✓ *Scientific Institute Public Health, Belgium*
- ✓ *Slovak Radiological Society. Slovakia*
- ✓ *SWEET e.V., Germany*
- ✓ *The Centre for Disease Prevention and Control, Latvia*
- ✓ *University Medical Center Hamburg-Eppendorf. Germany*
- ✓ *University of Glasgow, United Kingdom*

2. Health professionals' organisations (29)

- ✓ *Association of Speech Language Pathologists Malta*
- ✓ *Centre de Références des Infections Ostéoarticulaires Complexes (CRIOA)/Referral Centre for Complex Bone and Joint Infections, France*
- ✓ *Centres de référence des infections ostéoarticulaires graves et complexes (CRIOA)/Regional Referral Center for Complex Bone and Joint Infections, France*
- ✓ *EuroDSD Consortium*
- ✓ *European Board of Urology, EBU (Urology section of the UEMS)*
- ✓ *European Federation of Nurses Associations*
- ✓ *European Region of the World Confederation for Physical Therapy (ER-WCPT)*
- ✓ *European Society for Medical Oncology*
- ✓ *European Society of Cardiology*
- ✓ *FMA Finland*

- ✓ *Hellenic Society for the study of inborn errors of metabolism (HSSIEM), Greece*
- ✓ *Hospital Carlos Haya. Malaga. España*
- ✓ *Hospital Universitario Fundación Alcorcón, Spain*
- ✓ *INCCI (National Heart Institute in Luxembourg)*
- ✓ *IVAA International Federation of Anthroposophic Medical Associations*
- ✓ *Lithuanian Child Neurology Association*
- ✓ *Lithuanian Society for Paediatric Gastroenterology and Nutrition*
- ✓ *Lithuanian society of pediatric oncology and hematology*
- ✓ *Malta Association of Physiotherapists*
- ✓ *Ordem dos Enfermeiros, Portugal*
- ✓ *Paediatric Rheumatology International Trials Organisation (PRINTO at http://www.printo.it or www.pediatric-rheumatology.printo.it*
- ✓ *Pediatric Orthopaedic Unit. National Reference Unit in pediatric orthopedics. CSUR. Orthopedic Surgery and Traumatology. Faculty of Medicine. University of Murcia. University Hospital "Virgen de la Arrixaca". Department of Health. Murcia Health Service. Spain.*
- ✓ *Polish Nurses Association*
- ✓ *Radboud University Nijmegen Medical Centre, department of Neurology, Netherlands*
- ✓ *Servicio de Neurocirugía Pediátrica. Hospital Universitario 12 de octubre, Spain*
- ✓ *Slovenian Association of Radiology*
- ✓ *The European Glaucoma Society*
- ✓ *The Standing Committee of European Doctors (CPME)*
- ✓ *UEMS. Section/ European Board and College of Obstetrics and Gynaecology*

3. Patient organisation/association (26)

- ✓ *APDP - Associação Protectora dos Diabéticos de Portugal*
- ✓ *Asociacion de pacientes de Hemocromatosis (AEH). Spanish Hemochromatosis Patient Association*
- ✓ *Associação Portuguesa de Hemocromatose*
- ✓ *AZZURRA Associazione Malattie Rare Onlus, Italy*
- ✓ *Brittle Bone Society*
- ✓ *Cyprus Federation of Associations of Patients and Friends*
- ✓ *Cystic Fibrosis Europe (CFE)*

- ✓ *DEBRA International*
- ✓ *EFAPH – European Federation of Associations of Patients with Haemochromatosis*
- ✓ *EFAPH European Federation of Associations of Patients with Haemochromatosis*
- ✓ *EURORDIS – The European Organisation for Rare Diseases*
- ✓ *Hoffnungsbaum e.V. Verein zur Förderung der Erforschung und Behandlung von NBIA (vormals: Hallervorden-Spatz-Syndrom), in English: Tree of hope, Association dedicated to foster research and treatment in NBIA (formerly: Hallervorden-Spatz-Syndrome), ACRONYM: HoBa*
- ✓ *International Federation for Spina Bifida and Hydrocephalus*
- ✓ *International Federation for Spina Bifida and Hydrocephalus (IF)*
- ✓ *International Patient Organisation for Primary Immunodeficiencies (IPOPI)*
- ✓ *Krikos Zois (=LIFE-LINK) Greek Society for Patients and Friends of Patients with Inherited Metabolic Diseases*
- ✓ *Lithuanian association of people with genetic neuromuscular diseases "Sraunija"*
- ✓ *Madrid Spina Bífida Association (Asociación Madrileña de Espina Bífida), Spain*
- ✓ *Malta Health Network*
- ✓ *MITOCON insieme per lo studio e la cura delle malattie mitocondriali ONLUS, Italy*
- ✓ *PHA EUROPE - European Pulmonary Hypertension Association*
- ✓ *Rare Disorders Denmark*
- ✓ *SHINE- Spina Bifida.Hydrocephalus.Information.Networking.Equality*
- ✓ *The ESPKU -The European Society for Phenylketonuria and allied disorders*
- ✓ *Udruga osoba i roditelja djece sa spinom bifidom "Aurora", Croatia*
- ✓ *VSH vzw, Belgium*

4. ***Healthcare provider (23)***

- ✓ *AZ Vesalius, Belgium*
- ✓ *Department of Clinical Genetics, University and Regional Laboratories, Region Skane, Lund, Sweden*
- ✓ *Department of Medical Biochemistry, Oslo University Hospital, Rikshospitalet, Oslo Norway*
- ✓ *Department of Medical Genetics, Medical School, University of Athens, Greece*
- ✓ *Department of Paediatric Neurology, Pendeli Children's Hospital, Athens, Greece*
- ✓ *Department of Pathology, University and Regional Laboratories, Region Skåne, Sweden*

- ✓ *Division of Inherited Metabolic Diseases, Centre for Childhood and Adolescent Medicine, University Hospital Heidelberg, Germany*
- ✓ *Groupe MGEN - Mutuelle Générale de l'Education Nationale, France*
- ✓ *Hemochromatosis Clinic at Santo António Hospital, Portugal*
- ✓ *Hospital General Universitario Gregorio Marañón, Spain*
- ✓ *Hospital Universitario de Getafe (Getafe Universitary Hospital), Spain*
- ✓ *Kath. Kinderkrankenhaus Wilhelmstift gGmbH (Catholic Children's Hospital Wilhelmstift charitable non-proft Ltd), Germany*
- ✓ *Klinikverbund der geseztlichen Unfallversicherung e.V. (association of clinics of the German Social Accident Insurance, KUV), Germany*
- ✓ *Multidisciplinary Endocrine Tumor Team, Skåne University Hospital, Lund, Sweden*
- ✓ *North German Epilepsy Center of Hamburg*
- ✓ *Openbaar Psychiatrisch Zorgcentrum Rekem, Belgium*
- ✓ *Phakomatoses Centre, Hospital of the Lithuanian University of Health Sciences Kauno klinikos*
- ✓ *Rehabilitation and MS-Centre Overpelt, Belgium*
- ✓ *Riga Centre of Psychiatry and Addiction Medicine, Latvia*
- ✓ *SB SOFT Italy*
- ✓ *Scandinavian Ear Reconstruction Centre, Dept of Plastic and Reconstructive Surgery,*
- ✓ *Schoen Kliniken group, Schoen Klinik Hamburg Eilbek, Dept. of neurology and neurorehabilitation, Germany*
- ✓ *VZW Jessa Ziekenhuis, Belgium*

5. Healthcare provider organisations (7)

- ✓ *Central Manchester University Hospitals NHS Foundation Trust, UK*
- ✓ *European Chiropractors' Union (ECU)*
- ✓ *HospiLim, Belgium*
- ✓ *Royal Free london NHS Foundation Trust, UK*
- ✓ *Unit for Patient Safety Capital Region of Denmark*
- ✓ *Verband der Privatkrankenanstalten Österreichs, Austria*
- ✓ *Zorgnet Vlaanderen and ICURO, Belgium*

6. ***Public authorities and government-appointed bodies (13)***

- ✓ *Castilla y Leon Regional Health Service (Gerencia Regional de Salud de Castilla y Leon), Spain*
- ✓ *Directorate-General of Health / Ministry of Health, Portugal*
- ✓ *Entidade Reguladora da Saúde – ERS (Portuguese Health Regulatory Authority),*
- ✓ *Fundacion Rioja Salud, Spain*
- ✓ *Health Department, Campania Region - Italy*
- ✓ *Italian Technical Board of Regions for Rare Diseases*
- ✓ *Kassenärztliche Vereinigung Hamburg, KVH, Germany*
- ✓ *Ministère des Affaires Sociales et de la Santé, France*
- ✓ *Ministry for Health, the Elderly and Community Care, Malta*
- ✓ *Stowarzyszenie Chorych z Przepukliną Oponowo - Rdzeniową RP in Katowice, Poland*
- ✓ *The Norwegian Ministry of Health and Care Services*
- ✓ *VRCCRD - Veneto Region Coordinating Centre for Rare Diseases, Italy*
- ✓ *VSOP, Lupus Netherlands)*

7. ***Health insurer (2)***

- ✓ *Deutsche Gesetzliche Unfallversicherung (German Social Accident Insurance, DGUV)*
- ✓ *Landsbond van Onafhankelijke Ziekenfondsen - Union Nationale des Mutualités Libres, Belgium*

8. ***Individual respondent (5)***

- ✓ *Applus-novotec, Spain*
- ✓ *Assistance Publique Hôpitaux de Paris - Unité de Génétique des Maladies Cardiaques et Musculaires, France*
- ✓ *Dr mayka sanchez, Spain*
- ✓ *Foundation for Christianity, Hungary (2 contributions)*

Annex 2: Overview of comments/suggestions replied to open questions

All the individual comments are listed for each of the open questions included in the survey, with the wording provided by the respondents. The individual reports, accessible on the website, include all the comments made by each of the respondents.

A. Overview of free-style comments received Question 3.1 'Criteria related with diseases or conditions in order to be considered under the scope of the ERN

a.1. Recommended additional criteria or option that would effectively address the issue

Respondents were asked to recommend additional criteria or option that would effectively address the issue. Many valuable comments and suggestions were made. As a general remark, it can be concluded that most of the respondents pointed out that ERNs should focus on complex, highly specialised and rare diseases for which expertise is scarce.

The respondents indicated a number of key criteria which could be used for identifying the priority areas and conditions to be addressed, such as:

- Highly specialised and complex healthcare
- Multidisciplinary approach
- Continuity of care
- Continuity of expertise
- Comprehensive healthcare
- Concentration and co-ordination of research
- Exchange of knowledge and training
- Expertise and resources
- Prompt medical treatment
- Deeper evaluation of diagnosis and the therapy procedures
- Evidence-based activity
- After treatment social implications
- Financial support
- Patient-oriented activity
- Promotion of clinical trials
- ERNs organised by group of RD, etc.

Listed below are all the free comments that were made.

- ✓ Complexité des cas à traiter est un critère important, à côté de la rareté des cas. L'expertise spécialisée doit être multidisciplinaire. Voir réponse en papier.
- ✓ We recommend these additional criteria: Concentration and co-ordination of research related to the ERN team. Centre for concentration of specialist training.
- ✓ A particular aspect of the management of severely injured patients is urgent treatment with intermeshing of the rescue systems of primary shock therapy and of immediate operative and intensive therapy
- ✓ Prompt and comprehensive medical treatment (intermeshing of primary shock therapy, immediate operative and intensive therapy) for severely injured patients.
- ✓ Reference network centres should aim at further evaluation of diagnosis and therapy procedures, which are frequently absent
- ✓ Chronic condition with need of continuous interdisciplinary comprehensive health care, in-hospital & ambulatory, manifesting in different age groups with need of optimal transition between age groups.
- ✓ Evidence of scientific activity
- ✓ Additional criteria should be focusing on social aspects of treatment results and their impact on reintegration into working and social life.
- ✓ Financial support of the economically deprived countries with poor infrastructure for diagnosis and treatment of inherited metabolic disorders
- ✓ ERN organized per groups of RD with common diagnostic and/or care problems
- ✓ Particular aspect of the management of severely injured patients is urgent treatment, with intermeshing of the rescue systems of primary shock therapy and of immediate operative and intensive therapy
- ✓ ERN should be organized for groups of related RD rather than for single conditions
- ✓ We recommend these additional criteria: - Concentration and co-ordination of research related to the ERN team - Centre for concentration of specialist training
- ✓ Prompt and comprehensive medical treatment (intermeshing of primary shock therapy, immediate operative and intensive therapy) for severely injured patients
- ✓ Area of low EU health care workforce (as example more conservative spinal health care specialists) Emphasizing knowledge based intervention where this approach is equal or sup to hi-tech approach
- ✓ - testing of new drugs/treatment for rare diseases => better if same centres do this - when treatment has to start within certain time limit (f.e. stroke unit) => faster in hospital other side border
- ✓ Any European Reference Centre should be patient oriented, and prove this by a written concept, and collaborate with the competent European and national patient organisations

- ✓ Transparency to expertise and past performance
- ✓ Focus is transitional medicine as several complex diseases requiring highly specialized healthcare impose interdisciplinary challenges to both paediatric and adult medicine specialties
- ✓ In oncology, networks at least 1) share scarce expertise on rare cancers with high-level ordinary oncology units 2) widely share high-tech facilities 3) share locally ordinary expertise and facilities
- ✓ Multidisciplinary setting transition from paediatric care to adult services (change of type of service + other priorities for persons with disabilities) need for focus on ageing with SBH
- ✓ It would be wise to take into consideration research and teaching facilities and capabilities
- ✓ Need for a multidisciplinary approach, translational research
- ✓ Duration and continuity of expertise
- ✓ Would be recognized by national and/or EU level centre / providers Evidence sharing information, objective research and reported outcome
- ✓ Link to Patients' Organizations
- ✓ Concentration of resources and expensive infrastructure is crucial so other hospitals do not need to make investments in specific tools. Concentration of expertise in very important for rare diseases.
- ✓ We would like to highlight the need for highly-specialised health-care professionals to deal with patients and the problems associated with spina bifida and hydrocephalus. Expertise is lacking.
- ✓ Focus on ultra-rare diseases (low prevalence + scarce or not widely spread expertise) - goal: to concentrate and share expertise, rather than to provoke competition among hospitals
- ✓ Develop Research on the area of expertise
- ✓ Early genetic diagnostic is essential in some rare diseases, in our case is very important to prevent disease development. There is the need for specialized genetic/clinical diagnostic centres
- ✓ - collaboration in the definition of the targeted diseases and in establishing the rules for auditing the selected centres - JCI “quality label" (Joint Commission International) to be extended to EU
- ✓ Active research and post-graduate training in the field of expertise.
- ✓ Need for highly specialised HC professionals in the field of Spina Bifida and Hydrocephalus are necessary to gather scarce expertise within ERNs
- ✓ Risk of developing co-morbidities if not well treated -Need of long-term (life-long) follow-up and very regular check-up -Need of multi-disciplinary expertise for appropriate care and treatment
- ✓ Need of highly specialized HC professionals in the field of spina bifida and hydrocephalus are necessary to gather scarce expertise within ERNs
- ✓ Medicines frequently used off label/unlicensed in children; promoting clinical trials to enhance evidence of safe/effective therapies to be encouraged with adequate support for required infrastructure

- ✓ Main criteria are need for highly specialised care, medical equipment, need for concentration of resources and rare expertise
- ✓ EFN Position on Continuity of Care (http://www.efnweb.be/?page_id) Magnet Hospitals (http://journals.lww.com/jonajournal/Abstract/2011/10000/Nurse_Outcomes_in_Magnet__and_Non_Magnet_Hospitals.9.aspx)
- ✓ 1) Raise awareness particularly of GPs and Health Authorities on the importance of an early diagnosis 2) collect epidemiological data currently insufficient (reply EC E-012656/2011) and research
- ✓ The ERN should focus on highly available healthcare for all European citizens. We mean: economic, knowledge, and territorial availability. Highly inclusive for all, and for disabled in particular.
- ✓ Need for assessed competency in training specialists in the medical fields, as well as maintaining their competencies and sharing the knowledge across the continent.
- ✓ Inclusion in an international network
- ✓ See again proposed paediatric networks criteria at Ruperto N et al. A European network of paediatric research at the European Medicines Agency (Enpr-EMA). Archives Dis Child 2012; 97(3):185-188.
- ✓ Education and training, sharing of knowledge
- ✓ In case of rarity a joint approach of research and care is needed. By medical care you get data for research which might improve the care. Transition from paediatric to adult care should be addressed.
- ✓ I put 3 for all because it sounds not clear to me what an excellence centre is. Here we are not dealing with microbes but with people. Something else than cost-effective, risk benefit, cost, equipment
- ✓ Need for advanced/highly specialised HC professionals. Highly specialised professionals in the field of the disease or group of diseases concerned are necessary to gather scarce expertise within ERNs
- ✓ Table 1 of the Proposal: - is leading in the newest treatments and laboratory tests, also leading in the tests of new medicines the reference network should accept Patients' Rights act European model
- ✓ Possibility of disease prevention and cure or efficient treatment available if early diagnostic is done should be taken into consideration
- ✓ Focus should be given to rare diseases for which expertise is scarce. Treatable rare diseases (ie PIDs) should be a priority area. If diagnosed and treated early most PID patients lead normal lives
- ✓ Evaluation of clinical outcomes in referring centers. Setting criteria to evaluate results.

a.2. Prioritised/suggested concrete disease or group of disease to be addressed by the future ERN according to the above mentioned criteria

Respondents were asked to suggest concrete diseases or group of diseases to be addressed by the future ERN according to the above suggested additional criteria. Many concrete examples were provided.

Proposals can be classified in two main categories:

- Low prevalence/incidence and rare and complex diseases

- A list including a broad range of conditions including chronic, infectious, acute and other not defined conditions or age related diseases.

- ***Low prevalence/incidence and rare and complex diseases***

- ✓ Maladies rares et certains cancers constituent des domaines dans lesquels les RER sont très attendus et les plus faciles à imaginer. (Voir plus des détails dans la réponse en papier).
- ✓ Rare diseases
- ✓ Rare skeletal dysplasia. We have the most relevant collection in the world of DNA and clinical data for multiple osteocondromas and we treat also Osteogenesi Imperfecta, Enhler Duhnlos Syndrome etc.
- ✓ Rare neurodegenerative diseases
- ✓ The rare anaemia's group of diseases
- ✓ Rare cancer ERNs should have high priority because expertise on rare diseases is scarce. They should group together Centres of Excellence and high-quality general oncology facilities
- ✓ Epilepsy and other Seizure Disorders
- ✓ Spina bifida, hydrocephalus and related conditions.
- ✓ Lysosomal storage diseases, other rare metabolic disorders.
- ✓ Hemochromatosis and other rare iron overload diseases of genetic origin
- ✓ Autoinflammatory diseases (e.g. FMF, TRAPS, HIDS, CAPS, Schnitzler syndrome)
- ✓ Genodermatoses
- ✓ Rare and/or genetic disordes (e.g. mitochondrial diseases, congenital myasthenic syndromes)
- ✓ Primary Immunodeficiencies
- ✓ Rare genetic endocrine disorders.
- ✓ Disorders of Sex Development

- ✓ Different groups of inherited Metabolic Disorders e.g. Lysosomal Storage Diseases, Peroxisomal Leykodystrophiews, Congenital Disorders of glycosylation
- ✓ Tumour surgery, DBS or other functional surgery for PD and other movement disorders or epilepsy.
- ✓ Pain therapy. ITB for spasticity or dystonia. Medical therapy for rare disease. Cerebrovascular disease.
- ✓ Advancing novel diagnostics/treatment of, eg, autoimmune liver diseases, paediatric lysosomal and metabolic diseases, complex neurovascular and neuroimmunological disorders, neurofibromatosis
- ✓ Inherited Metabolic Disorders(IEM): Lysosomal Storage Diseases, Peroxisomal Leukodystrophies, Congenital Disorders of Glycosylation etc., fulfil the ERN scope and require clinical-lab networking
- ✓ Prosthetic joint infections
- ✓ Prosthetic vascular graft infection
- ✓ Paediatrics' transplants
- ✓ Children hand surgery + children craniofacial surgery in a for children suitable environment Epidermolysis bullosa
- ✓ Hadron-therapy in Cancer
- ✓ Discrepancies growth, bone dysplasias, bone reconstruction
- ✓ Spinal Surgery, paediatric hip-knee-ankle prosthesis, serious infectious bone diseases,
- ✓ Foot and shoulder rare diseases, regenerative Medicine, muscoloskeletal tissue bank
- ✓ Central Nervous System (CNS) Oncology Congenital CNS Malformations including Craniofacial surgery Epilepsy surgery
- ✓ Multiple Sclerosis
- ✓ Tuberous sclerosis, Neurofibromatosis type I-II, Neuromuscular disorders, Myelomeningocoelle, Leucodystrophies, Childhood multiple sclerosis or Childhood autoimmune neurological disorders
- ✓ Phakomatoses (Tuberous sclerosis, Neurofibromatosis type I-II), Myelomeningocoelle, Neuromuscular disorders, Childhood multiple sclerosis.
- ✓ Endocrine tumors (ET) and radionuclide therapies (RNT). Most types of ET are rare, and there are few specialists in the field. RNT is a resource-intensive modality, centralization for cost-efficiency.
- ✓ Microtia
- ✓ High specialised urologic conditions.
- ✓ Primary rare glaucoma conditions such as congenital glaucoma
- ✓ Neuro-oncology, cerebrovascular, functional neurosurgery and skull base neurosurgery.
- ✓ Pain therapy. ITB for spasticity or dystonia
- ✓ Transplants (liver, pancreas)
- ✓ Neurodegenerative disorders involving motor neuron disease and white matter.
- ✓ Fragile x syndrome Childhood neurodevelopmental disorders

- ***Chronic, infectious, acute and other not low prevalence or not defined conditions or age related diseases***

- ✓ For all diseases
- ✓ Paediatric diseases in general
- ✓ Non-communicable diseases (including cardiovascular disease),
- ✓ Diabetes/NCDs
- ✓ Infectious Diseases
- ✓ AIDS, tuberculosis and malaria due to migratory fluxes from non-EU Countries.
- ✓ ERN for comprehensive treatment of accident victims: treatment of acute injuries, early rehabilitation, seamless transition to full rehabilitation, performance of reconstructive procedures
- ✓ Burns for capacity issues
- ✓ Stroke units in cross border areas -
- ✓ Lung carcinoma
- ✓ Cerebral stroke hypoxemic encephalopathy dementia
- ✓ Treatment of coronary disease, acute stroke, orthopaedics and oncological diseases – High prevalence in community. –
- ✓ Transplants (kidney)
- ✓ Cerebrovascular disease.
- ✓ Carcinoma mammae
- ✓ Cancer
- ✓ Chronic diseases, especially those with high risk/rate of co-morbidities if not treated well, such as diabetes.
- ✓ Group of diseases linked to the ageing of the population
- ✓ Hepatological diseases
- ✓ Inclusion of MSK-conditions
- ✓ Cardiovascular diseases-certain cases cancers neurological diseases
- ✓ Primary (congenital) and secondary (after cancer treatment) arm and leg lymphedema.
- ✓ Behavioural disorders
- ✓ All Orthopaedic Surgery
- ✓ RG3agents; Bacterial enter m/o;C.diphtheriae; Staph.-Streptococci; Meningococci; VHA,B,C,D,E; HIV; Influenza; Polio and other Enteroviruses; Measles, Rubella, Mump; TBE, Borrelia; Herpes; viral gastroenteritis

B. Overview of free-style comments received on Question 3.2 'General criteria of the centres wishing to join a European Reference Network

b.1. Recommended additional option that would effectively address the issue

- ✓ Pour les autorités françaises il importe que le centre de référence adhère à sa dimension européenne et qu'il informe les autorités nationales de sa démarche, dans une logique de cohérence.
- ✓ The centre should be a referral centre at the local level.
- ✓ Development of process and communication structures to appropriately follow up of the patients progress
- ✓ Cross-cultural communication and foreign language competences.
- ✓ Involvement of Regional Health Authorities, through regional/interregional coordinating Centres, where established per Law, as they are formally in charge to support and monitor CoE RDrelated activities
- ✓ Specific and mandatory involvement of Regional Health Authorities, to which CoE formally respond for their activities
- ✓ The centre should be a referral centre at the local level.
- ✓ Overall capacity and commitment to the management of the area of interest. Pan-European organization Professions with a common educational standard. Pursuing clinical excellence Cost-effectiveness
- ✓ Research possibilities, high quality, patient centred care are the most important criteria
- ✓ Geographic location/accessibility, multidisciplinary health care and patient focus, including support to patients' families and accompanying persons.
- ✓ - the key ambition should be: share expertise and give/get coaching/training - once a patient is back in home: the Belgian specialist must be able to ask info to foreign provider , ERN as back office
- ✓ High level of clinical expertise, patients empowerment, psychological skills and resources, HTA studies, e-health: Gephcard for clinical and genetic data collection and LabRER for quality government
- ✓ Structured follow-up care programs für HSHC patients warranting quality management and evaluation of clinical pathways, transcultural patient care and foreign language competence for cross-border care
- ✓ A 'network patient' should be identified, since not all patients treated at participating centers will benefit from network quality system; criteria shouldn't pose excessive admin/formal burdens
- ✓ involvement of DPO's (disabled persons organisations) in the implementation of services and in the ERN
- ✓ interdisciplinary as well as stationary and ambulatory cooperation
- ✓ Evidence based practice, cost-effectiveness, outcome
- ✓ Limiting the minimum number of cases available at the CoE would not allow small countries to join ant network ever therefore should not be a criteria for CoE.

- ✓ Setting the limits for the minimum number of patients for the CoE as criteria is not appropriate as this would not allow small countries to join the network - the ones that badly need networking!
- ✓ It is important that not only large hospitals participate in the reference networks but that there is also the possibility for smaller hospitals to take part.
- ✓ We also want general hospitals to be taken into account joining an ERN, not only University hospitals. Clinical research is very important. Patient must be central. Translators must be available.
- ✓ People with spina bifida and/or hydrocephalus and their relatives should be involved in running centres of excellence, in line with the "nothing about us without us" ethos.
- ✓ - data registration + actual expertise sharing is crucial - expertise is the most significant criterion - financial stability (to exclude economic motives) -interpreters for foreign patients
- ✓ Existing connexion with colleagues of the same speciality from other countries
- ✓ Expertise based in research activities, training and full commitment
- ✓ - JCI “quality label" (Joint Commission International) to be extended to
- ✓ Involvement of POs & patient representatives in the governance/activities of the CoE; ability to contribute and implement good practice guidelines; ability to collect data for registries
- ✓ -Critical amount of patients treated on yearly basis -Presence of multidisciplinary team -Collaboration with other expert centres & with patient associations -System to ensure cross-border healthcare
- ✓ involvement of POs and patient representatives in the governance/activities of the Coe; ability to contribute and implement good practice guidelines, ability to collect data for registries
- ✓ to demonstrate effectiveness and expertise such as required by Enpr-EMA recognition criteria http://www.ema.europa.eu/docs/en_GB/document_library/Template_or_form/2010/02/WC500073674.doc
- ✓ Involvement into international clinical trials on specific diseases.
- ✓ Acceptance to share data and biological samples with the network partners so that setting up highly specialized techniques would not be necessary for every partner of the network
- ✓ EFN Position Statement on Criteria for Excellence Centres (http://www.efnweb.be/?page_id=845).
- ✓ Involvement into international clinical trials on specific diseases.
- ✓ Lymphedema induces adipose tissue hypertrophy. Our treatment with liposuction is the only one which can completely normalize chronic large non-pitting lymphedemas.
- ✓ Geographical location is a critical issue: frontier locations (for example Southern Italy) are more exposed to the recrudescence of some specific diseases (i.e. from Africa and eastern EU).
- ✓ Biological research should not be necessary to join an ERN but ability to perform clinical and therapeutical research is important.

- ✓ Sharing of knowledge (arranging courses, communicating with other health care providers and the public etc.)
- ✓ willingness, abilities and facilities of the centre to contribute to psychosocial and/or palliative care for patients
- ✓ Involvement of POs & patient representatives in the governance/activities of the CoE; ability to contribute and implement good practice guidelines; ability to collect data for registries
- ✓ Strong patient representation in the management of the ERN and the development of strategy. Integration with social care services.
- ✓ ability to monitor patients at a distance, good network of transportation
- ✓ National (bio and gen) database exchange and international communication features
- ✓ HTA assessment for RD such as PIDs should take into account the significant macro-economic and societal benefits which early diagnosis and appropriate treatment will bring to patients

C. Overview of free-style comments received on Question 3.3 'Specific criteria regarding the areas of expertise'

c.1. Recommended additional criteria/option that would effectively address the issue

- ✓ Yes: trauma register for monitoring of the quality of treatment and for comparison between participating institutions.
- ✓ Research for rare disease like mesothelioma and asbestosis. Development and update of guidelines for the diagnosis and therapy of these diseases.
- ✓ Implementation of trauma registers for monitoring quality treatment and for comparison between the participating institutions. Research in the field of mesothelioma/asbestosis including guidelines
- ✓ involved in JCI accreditation and HTA finalized to improve clinical practice
- ✓ Development of standards and protocols of care with special emphasis on follow-up assessment and long-term care management.
- ✓ The Spanish experience could be useful.
- ✓ The RCCRD considers a priority the organization of an UE Registry of RDs as well as the creation of a EU network of Lysosomal Diseases Centre of Excellence
- ✓ Structural preconditions for communication with patients and providers of the countries of origin are to be established.
- ✓ Pulmonary arterial hypertension is one particular field in which the designation of CoEs and ERN is critical as it is very complex, rare disease requiring concentration of expertise.
- ✓ Trauma registers for monitoring of the quality of treatment and for comparison between participating institutions
- ✓ Connecting CoE with the other nodes of the care network; tele-consultation; outcomes objectively pre-evaluated by the competent Regional Health Authorities
- ✓ Malta feels that the criteria mentioned in 3.3.1 above are the key criteria to effectively address this issue.
- ✓ Implementation of a trauma registers to monitor quality and to compare, research in the field of mesothelioma/asbestosis including introduction of guidelines for diagnosis and therapy
- ✓ National or supranational data-collection of outcomes (Service quality-measuring patient outcomes and satisfaction with Care Response) Multidisciplinary care
- ✓ Possibility of multidisciplinary team based care is important, with good clinical pathways and research based medicine (evidence based medicine)

- ✓ Need for evaluation by independent entity (no auto-proclamation) Need for objective criteria for selection (no political criteria) Hospitals need to be accredited.
- ✓ We could contribute with: • highly competences, experience and good outcomes and care. • psychological resources on patients empowerment • Next Generation sequencing • E-health innovative IT solution
- ✓ In our point of view, genetics, It is a good tool to diagnosis of our patients.
- ✓ I think it would be important not to assign the ERN status through a top-down approach, but to let centres apply.
- ✓ The consideration of ethical and legal issues for each member state
- ✓ Integration of follow-up programs in clinical pathways, evaluation/quality management to achieve treatment and research goals, bridging the gap between children and adults (transitional medicine)
- ✓ ERNs should be cancer-specific, inasmuch as they should 'own' all diagnostic and therapeutic options needed in the disease. A certification system should be in place.
- ✓ Multidisciplinary setting, attention to transition in services, ageing of the addressed population, ability to gather a critical mass
- ✓ Good communications networks and standards are essential to support this work
- ✓ Physical space within a hospital for specialised care (rooms)
- ✓ As a UK charity specialising in advocacy and support for those with a rare condition like OI it is imperative we build strong links with healthcare professionals in centres of excellence.
- ✓ Standardised and organised knowledge exchange
- ✓ International Links (with other reference centres)
- ✓ General remark: There is a trend for measuring quality by looking at the number of patients that one doctor treats, however this is not always the right way to assess quality.
- ✓ Very important to ensure people with spina bifida and/hydrocephalus are engaged in centres of excellence as they have personal experience of the condition and enjoy the right to run their own affairs.
- ✓ Medical expertise is the most important criterion, given the focus on ultra-rare diseases.
- ✓ ERN governance, interaction of members, financial support if any should be clearly defined. Adjustments depending on the scope of ERN possible, e.g. laboratory networks for IEM
- ✓ Pre-existing collaboration of the health care provider with other health care provider or a network with the same interest both on the clinical and research front is important
- ✓ Link to Patients Associations
- ✓ Including in ENR related health professionals such as Speech Language Pathologists/Speech Therapists/ Logopaedists either from national organisations or in consultation with CPLOL.

- ✓ With regards to Patient representation there should be a contact point in each European country possibly coming/appointed from a recognized /registered umbrella organisation for fair representation
- ✓ Facility for inter-European collaboration
- ✓ Establishment of specific clinical consultations for patients. International coordination with other centres.
- ✓ Overall multi-disciplinarily, including clinical and social/supportive care, flexibility of the structure, interoperability, training & information, ability to gather critical mass of patients & data
- ✓ Epidemiological surveillance (e.g. benchmarking, defining best practices,…) -Specific arrangements for patient referrals from other EU counties -Health economic analysis -Disease specific registry
- ✓ Overall multidisciplinary, including clinical and social/supportive care, flexibility of the structure, interoperability, training and information, ability to gather critical mass of patients and data
- ✓ Evidence of training and updating GCP should be provided. Unit centres and networks should be involved in research and trials if they aspire to clinical excellence and leadership.
- ✓ Most important criteria are: - competence, record of good outcomes; - regular use of agreed protocols and clinical guidelines; - commitment to research
- ✓ The key points are clinical experience, organisation, motivation, acceptance for strong communication and exchanges, acceptance to resort to specific diagnostic tools in the hands of other partners
- ✓ Evidence based care pathways and guidelines, nursing leadership and research opportunities, multidisciplinary approaches, optimal staffing skill mix ratios.
- ✓ Stability of funding
- ✓ We would be highly interested in applying to the process to be considered Centre of Excellence of the future European Reference Network
- ✓ Expertise, protocols, best practice and disease registers should be shared through a common ICT platform to improve the performances of all member States.
- ✓ For us it is very important that the surgical treatment of microtia (ear deformities) is concentrated to limited number of centres of excellence in Europe.
- ✓ Telemedicine
- ✓ The creation of a European network of units generates higher quality in the treatment of rare diseases. Our patients will benefit from this European coordination.
- ✓ Given the prominent role the CoEs will have for coordinating care, disseminating knowledge and training we believe all should be committed to implementing a robust quality and safety system.
- ✓ EBCOG maintains that keeping the highest standards of training of specialist and the highest standards of care within the speciality is of utmost importance in the European medical scene of to-morrow

- ✓ Research for rare disease like mesothelioma or asbestosis. Development and update of guidelines for the diagnosis and therapy of these diseases.
- ✓ Ability to participate to international cooperation projects.
- ✓ The idea of ERNs is excellent for rare diseases, but there is also a need for regional centres working with the ERNs to give patient access to daily care as close to home as possible
- ✓ Consider the peculiarity of paediatric research that needs by definition international large networks especially for the less prevalent childhood diseases
- ✓ Numbers of diagnosed/treated/advised patients per year or since a date at a CE, related to the prevalence of the disease
- ✓ For patients the centre should be convenient to reach
- ✓ No further comments at this point. If we discuss specific disease entities, I might come up with further comments.
- ✓ Good initiative.
- ✓ The establishment of highly specialised care also requires training pathways for healthcare professionals. These are often not yet available or show a significant variation in EU MS.
- ✓ Centre of excellence = also ... multidisciplinary, community of practices, human approach of patients, palliative care, nursing, ...
- ✓ Overall multi-disciplinarity, including clinical and social/supportive care, flexibility of the structure, interoperability, training & information, ability to gather critical mass of patients & data
- ✓ Integration with social care provision.
- ✓ Connection to formal education and research institutions
- ✓ Prevention of regression of the quality of life of patients should be an item!
- ✓ Efforts to support reference networks should be done in EU because some countries are dramatically cutting investment in health and research (Portugal Greece Spain)
- ✓ Effective patient registry. ESID registry provides a gold standard example of an EU wide PID registry to improve knowledge about the conditions, facilitate research and information exchange.
- ✓ I think that the European Commission could have a budget for meetings about specialized diseases, and that from this meetings of all interested persons, integrated actions could be planned and fulfil
- ✓ The most important factor is "human resources"
- ✓ Open to collaboration in the field of COEs and ERN Developing strategies in our institution to be designated
- ✓ Specific information system (not only expertise)

Annex 3: List of proposed criteria for ERNs (scope, general and specific criteria)

a.1. List of criteria related with diseases or conditions in order to be considered under the scope of the ERN		
1	Need of highly specialised healthcare	• Complexity of the diagnosis and treatment • High cost of treatment and resources • Need of advanced/highly specialised medical equipment or infrastructures
2	Need of particular concentration of expertise and resources	• Rare expertise/need of concentration of cases • Low prevalence/incidence/number of cases • Evaluated experiences of Member States
3	Based on high-quality, accessible and cost-effective healthcare	• Evidence of the safety and favourable risk-benefit analysis • Feasibility and evidence of the value and potential positive outcome (clinical)

a. 2. List of general criteria of the centres wishing to join a European Reference Network (*)	
1	Organisation and management
2	Patients empowerment and centred care
3	Patient care, clinical tools and health technology assessment
4	Quality, patient safety and evaluation framework policies
5	Business continuity, contingency planning and response capacity
6	Information systems, technology and e-health tools and applications
7	Overall framework and capacity for research and training
8	Specific commitment of the management/direction of the centre/hospital to ensure a full and active participation in the ERN

(*) Sub-criteria and quantitative or qualitative requisites will be further defined for each of the listed criteria.

a.3. List of specific criteria regarding the areas of expertise

1. Competence, experience and good outcomes and care
2. Specific resources and organisation
    a. Human resources
    b. Team/centre organisation
    c. Structural conditions
    d. Specific equipment
3. Presence and coordination with other required complementary units or services
4. Patient care pathways, protocols and clinical guideline in the field of expertise
5. External coordination, care management and follow-up of patients
6. Research, training, health technology assessment in the field of expertise
7. Specific information systems

Annex 4: Response statistics for Public consultation

1. Respondent Profile

1.1 Please indicate the type of organisation on behalf of which you are responding to this consultation:

	Number	% of (138)
Patient organisation/association	26	18,84%
Health professionals' organisations (e.g. doctors, nurses, managers)	29	21,01%
Healthcare provider organisations (e.g. hospitals association, specialised provider association)	7	5,07%
Healthcare provider	23	16,67%
Academic/public health and healthcare specialised institution/organisation (e.g. Institutes and University Departments of Public Health, Quality, Healthcare, Clinical Excellence)	33	23,91%
Healthcare public administration at national level	1	0,72%
Healthcare public administration at regional level	6	4,35%
Healthcare public administration at local level	0	0,00%
Public authorities and government-appointed bodies responsible or involved in the definition of criteria and the establishment and evaluation of centres of reference/excellence (e.g. highly specialised healthcare, specialised commissioning services)	6	4,35%
Health insurer (e.g. sickness fund)	2	1,45%
Individual respondent	5	3,62%

Please indicate level: (requested respondents: Patient organisation/Association, Health professionals organisation, Healthcare provider organisation, Academic/Public Health)

	Number	% of (95)
National level organisation	67	70,53%
European Union umbrella organisation	28	29,47%

Please indicate level: (requested respondents: Public authorities and government-appointed bodies)

	Number	% of (6)
National level	5	83,33%
Regional level	1	16,67%

Please indicate Member States representation: (requested respondents: “European Union umbrella organisation)

	Number	% of (28)
Less than 10 Member States	0	0,00%
Between 10-20 Member States	7	25,00%
Pan European	21	75,00%

Please indicate what type: (requested respondents: Healthcare provider)

	Number	% of (23)
Primary healthcare provider/centre	0	0,00%
Hospital	14	60,87%
In hospital specialised service or unit	8	34,78%
Ambulatory specialised service or unit	0	0,00%
Public/contracted centre	1	4,35%
Private not under contract	0	0,00%

Please indicate for what the administration is responsible: (requested respondents: Healthcare administration at national, regional and local level)		
	Number	**% of (7)**
Healthcare planning/organisation	2	28,57%
Public Health	1	14,29%
Health Technology Assessment	0	0,00%
Quality, patient safety or clinical governance	1	14,29%
National/regional health service provider	3	42,86%
Please indicate from which sector: (requested respondents: Health insurer)		
	Number	**% of (2)**
Public	2	100,00%
Private	0	0,00%
1.3 Please indicate the country where your organisation/centre is located/has its headquarters or main representative office in Europe:		
	Number	**% of (138)**
BE	19	13,77%
DK	2	1,45%
DE	15	10,87%
EL	6	4,35%
ES	15	10,87%
FR	13	9,42%
IT	12	8,70%
CY	1	0,72%
LV	3	2,17%
LT	7	5,07%
LU	1	0,72%
HU	2	1,45%
MT	4	2,90%
NL	4	2,90%
AT	3	2,17%
PL	2	1,45%
PT	6	4,35%
SI	1	0,72%
SK	1	0,72%
FI	1	0,72%
SE	6	4,35%
UK	9	6,52%
NO	3	2,17%
HR	2	1,45%
1.4 Please indicate the number of EU Member States and EEA countries (Norway, Iceland, Lichtenstein) and accessing country (Croatia) in which your organisation conducts business/is represented:		
	Number	**% of (138)**
1	73	52,90%
2	5	3,62%
3	5	3,62%
4	3	2,17%
5	3	2,17%
6	2	1,45%
7	1	0,72%
8	1	0,72%
10	5	3,62%
11	3	2,17%
12	2	1,45%

13	2	1,45%
15	2	1,45%
17	1	0,72%
18	2	1,45%
20	4	2,90%
22	1	0,72%
23	1	0,72%
24	2	1,45%
25	3	2,17%
26	2	1,45%
27	2	1,45%
28	2	1,45%
29	1	0,72%
31	10	7,25%

1.5 If need be, can we contact you by e-mail to obtain further information on your submission?

	Number	% of (138)
Yes	134	97,10%
No	4	2,90%

2. Involvement of your organisation in the matter of centres of excellence/reference (COE) and healthcare networks in highly specialised healthcare (HSHC).

2.1 How would you describe your organisation's knowledge of CoE and HSHC?

	Number	% of (138)
Very high	39	28,26%
High	61	44,20%
Poor	25	18,12%
None	2	1,45%
No opinion	6	4,35%
Non applicable	5	3,62%

2.2. What aspects or domains related to the topic of CoE and HSHC would correspond to your organisation's key knowledge? (cross any that applies)

	Number	% of (138)
Highly specialised healthcare provision	83	60,14%
Priorities, description and characteristics of CoE and HSHC	50	36,23%
Management and organisational aspects of highly specialized healthcare	64	46,38%
Professional performance, clinical practice, quality and safety of specialized healthcare	92	66,67%
Assessment/evaluation/certification of clinical practice and healthcare providers	57	41,30%
Information system, coding	28	20,29%
Engineering/e-health	20	14,49%
Costs and economic evaluation	21	15,22%
Ethical analysis	31	22,46%
Social aspects	41	29,71%
Legal aspects	21	15,22%
No opinion	1	0,72%
Non applicable	5	3,62%

2.3 Is highly specialised healthcare a priority in your organisation's strategies and work plans?		
	Number	**% of (138)**
Very high	71	51,45%
High	37	26,81%
Somewhat	21	15,22%
Low	4	2,90%
No priority	2	1,45%
No opinion	1	0,72%
Non applicable	2	1,45%

2.4. What specific field of healthcare services/specialities are most relevant for your centre/organisation's field of work?		
	Number	**% of (138)**
Surgical speciality	9	6,52%
Medical speciality	24	17,39%
Medical/Surgical speciality	30	21,74%
High tech diagnostic and treatment services (radiology, nuclear medecine, electrophysiology, radiotherapy etc.)	12	8,70%
Laboratories and central services (e.g. pathology, genetics, biochemistry, pharmacy, microbiology etc.)	22	15,94%
Other	30	21,74%
No opinion	3	2,17%
Non applicable	8	5,80%

2.5. Has your organisation/centre been directly involved in the design or assessment of professional standards and criteria related with highly specialised healthcare?		
	Number	**% of (138)**
Frequently	59	42,75%
Occasionally	50	36,23%
Never	21	15,22%
No opinion	4	2,90%
Non applicable	4	2,90%

2.6. Has your organisation been involved in projects/activities supported by the Commission in relation with HSHC or professional and technical criteria/standards in highly specialised healthcare?		
	Number	**% of (138)**
Yes	46	33,33%
No	81	58,70%
Non applicable	11	7,97%

2.7. Do you have concrete examples based on your own organisation's experience or could you provide us with references or links to documents related with professional criteria and standards in highly specialised healthcare/CoE or HSHC (e.g. quality criteria, guidelines, consensus documents)?		
	Number	**% of (138)**
Yes	90	65,22%
No	38	27,54%
Non applicable	10	7,25%

2.8. Is your centre or unit directly involved in the management (diagnosis, treatment etc.) of highly specialised diseases or conditions? (requested respondents: Healthcare provider)		
	Number	**% of (23)**
Yes	20	86,96%
No	1	4,35%
Non applicable	2	8,70%

2.9. Is your centre or unit designated or recognised as centre of reference/excellence in your country? (requested respondents: Healthcare provider)		
	Number	**% of (23)**
Yes	17	73,91%
No	2	8,70%
Non applicable	4	17,39%

2.10. How has your centre been designated/recognised as centre of reference/excellence? (requested respondents: Healthcare provider from designated/recognised centre of reference/excellence)		
	Number	**% of (17)**
Through a formal institutional process	7	41,18%
Based on professional recognition	7	41,18%
Other	3	17,65%

2.12 Is your centre participating currently in a network of centres of expertise? (requested respondents: Healthcare provider from designated/recognised centre of reference/excellence)		
	Number	**% of (17)**
Yes	15	88,24%
No	2	11,76%
Non applicable	0	0,00%

2.13. What is the scope of the network? (requested respondents: Healthcare provider from centre participating currently in a network of centres of expertise)		
	Number	**% of (15)**
Regional	1	6,67%
National	5	33,33%
International	9	60,00%

2.14. Which kind of network? (requested respondents: Healthcare provider from centre participating currently in a network of centres of expertise)		
	Number	**% of (15)**
National	6	40,00%
International	6	40,00%
Founded by the European Commission	1	6,67%
Founded by other sources	2	13,33%

2.15. Would you be interested in applying to the process to be considered Centre of Excellence of the future European Reference Network? (1 = not interested at all, 5 = very interested) (requested respondents: Healthcare provider from centre directly involved in the management of highly specialised diseases or conditions)		
	Number	**% of (20)**
1	0	0,00%
2	1	5,00%
3	1	5,00%
4	2	10,00%
5	16	80,00%

3. Proposed criteria for ERN (scope, general and specific criteria)

3.1 Criteria related with diseases or conditions in order to be considered under the scope of the ERN

3.1.1. Need of highly specialised healthcare		
	Number	**% of (138)**
1	3	2,17%
2	1	0,72%
3	4	2,90%
4	23	16,67%
5	107	77,54%

3.1.1.1. Complexity of the diagnosis and treatment		
	Number	**% of (138)**
1	1	0,72%
2	1	0,72%
3	8	5,80%
4	35	25,36%
5	93	67,39%
3.1.1.2. High cost of treatment and resources		
	Number	**% of (138)**
1	4	2,90%
2	13	9,42%
3	29	21,01%
4	38	27,54%
5	54	39,13%
3.1.1.3. Need of advanced/highly specialised medical equipment or infrastructures		
	Number	**% of (138)**
1	1	0,72%
2	7	5,07%
3	22	15,94%
4	43	31,16%
5	65	47,10%
3.1.2. Need of particular concentration of expertise and resources		
	Number	**% of (138)**
1	3	2,17%
2	0	0,00%
3	11	7,97%
4	26	18,84%
5	98	71,01%
3.1.2.1. Rare expertise/need of concentration of cases		
	Number	**% of (138)**
1	3	2,17%
2	3	2,17%
3	11	7,97%
4	29	21,01%
5	92	66,67%

3.1.2.2. Low prevalence/incidence/number of cases		
	Number	**% of (138)**
1	3	2,17%
2	4	2,90%
3	25	18,12%
4	42	30,43%
5	64	46,38%
3.1.2.3. Evaluated experiences of Member States		
	Number	**% of (138)**
1	1	0,72%
2	13	9,42%
3	36	26,09%
4	51	36,96%
5	37	26,81%

3.1.3. Based on high-quality, accessible and cost-effective healthcare		
	Number	**% of (138)**
1	3	2,17%
2	3	2,17%
3	11	7,97%
4	47	34,06%
5	74	53,62%
3.1.3.1. Evidence of the safety and favourable risk-benefit analysis		
	Number	**% of (138)**
1	1	0,72%
2	6	4,35%
3	24	17,39%
4	45	32,61%
5	62	44,93%
3.1.3.2. Feasibility and evidence of the value and potential positive outcome (clinical)		
	Number	**% of (138)**
1	1	0,72%
2	3	2,17%
3	17	12,32%
4	44	31,88%
5	73	52,90%
3.1.4. Do you recommend any additional criteria or option that would effectively address the issue?		
	Number	**% of (138)**
Yes	49	35,51%
No	89	64,49%
3.1.5. Would you prioritise or suggest any concrete disease or group of diseases to be addressed by the future ERN according to the above criteria?		
	Number	**% of (138)**
Yes	88	63,77%
No	50	36,23%

3.2. General criteria of the centres wishing to join a European Reference Network

3.2.1. Organisation and management

	Number	% of (138)
1	4	2,90%
2	3	2,17%
3	17	12,32%
4	51	36,96%
5	63	45,65%

3.2.2. Patients empowerment and centered care

	Number	% of (138)
1	3	2,17%
2	3	2,17%
3	8	5,80%
4	46	33,33%
5	78	56,52%

3.2.3. Patient care, clinical tools and health technology assessment

	Number	% of (138)
1	2	1,45%
2	2	1,45%
3	7	5,07%
4	45	32,61%
5	82	59,42%

3.2.4. Quality, patient safety and evaluation framework policies

	Number	% of (138)
1	2	1,45%
2	3	2,17%
3	6	4,35%
4	36	26,09%
5	91	65,94%

3.2.5. Business continuity, contingency planning and response capacity

	Number	% of (138)
1	1	0,72%
2	8	5,80%
3	33	23,91%
4	50	36,23%
5	46	33,33%

3.2.6. Information systems, technology and e-health tools and applications

	Number	% of (138)
1	2	1,45%
2	2	1,45%
3	26	18,84%
4	49	35,51%
5	59	42,75%

3.2.7. Overall framework and capacity for research and training		
	Number	**% of (138)**
1	3	2,17%
2	2	1,45%
3	11	7,97%
4	46	33,33%
5	76	55,07%

3.2.8. Specific commitment of the management/direction of the centre/hospital to ensure a full and active participation in the ERN		
	Number	**% (138)**
1	2	1,45%
2	3	2,17%
3	11	7,97%
4	44	31,88%
5	78	56,52%

3.2.9. Do you recommend any additional option that would effectively address the issue?		
	Number	**% of (138)**
Yes	39	28,26%
No	99	71,74%

3.3. Specific criteria regarding the areas of expertise

3.3.1. Competence, experience and good outcomes and care		
	Number	**% of (138)**
1	3	2,17%
2	0	0,00%
3	1	0,72%
4	15	10,87%
5	119	86,23%

3.3.2. Specific resources and organisation:		
	Number	**% of (138)**
1	3	2,17%
2	0	0,00%
3	14	10,14%
4	38	27,54%
5	83	60,14%

3.3.2.1. Human resources		
	Number	**% of (138)**
1	1	0,72%
2	3	2,17%
3	12	8,70%
4	32	23,19%
5	90	65,22%

3.3.2.2. Team/centre organisation		
	Number	**% of (138)**
1	2	1,45%
2	1	0,72%
3	10	7,25%
4	49	35,51%
5	76	55,07%

3.3.2.3. Structural conditions		
	Number	**% of (138)**
1	0	0,00%
2	1	0,72%
3	28	20,29%
4	72	52,17%
5	37	26,81%

3.3.2.4. Specific equipment		
	Number	**% of (138)**
1	0	0,00%
2	5	3,62%
3	16	11,59%
4	58	42,03%
5	59	42,75%

3.3.2.5. Presence and coordination with other required complementary units or services		
	Number	**% of (138)**
1	4	2,90%
2	2	1,45%
3	13	9,42%
4	38	27,54%
5	81	58,70%

3.3.3. Patient care pathways, protocols and clinical guidelines in the field of expertise		
	Number	**% of (138)**
1	3	2,17%
2	0	0,00%
3	6	4,35%
4	37	26,81%
5	92	66,67%

3.3.4. External coordination, care management and follow-up of patients		
	Number	**% of (138)**
1	1	0,72%
2	1	0,72%
3	10	7,25%
4	54	39,13%
5	72	52,17%

3.3.5. Research, training, health technology assessment in the field of expertise		
	Number	**% of (138)**
1	3	2,17%
2	2	1,45%
3	11	7,97%
4	45	32,61%
5	77	55,80%

3.3.6. Specific information systems		
	Number	**% of (138)**
1	2	1,45%
2	4	2,90%
3	25	18,12%
4	65	47,10%
5	42	30,43%

3.3.7. Do you recommend any additional criteria or option that would effectively address the issue?		
	Number	**% of (138)**
Yes	30	21,74%
No	108	78,26%