Informal FAQs and Discussions on RD ERNs

Based on the Workshop '*Realising Rare Disease European Reference Networks*' (1st and 2nd July 2015, Brussels)

and updated January 2016

Co-funded by
the Health Programme
of the European Union

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Introduction to the Informal FAQs

This document is intended to support the rare disease (RD) field in preparing for the first call for ERNs, by presenting and adapting the important discussion sessions from the expert workshop co-hosted by the EUCERD Joint Action for Rare Diseases (EJA)[1] and the European Commission (EC) in Summer 2015, and using these to crystallise frequently asked questions (FAQs) surrounding ERNs. The Policy work package of RD-ACTION[2] has continued to focus activities around the topic of RD ERNs in the aftermath of the workshop - an important step was to prepare a set of official FAQs[3] together with DG Santé, to clarify some of the issues surrounding (in particular) the organisational and procedural aspects of ERNs. However, in recognition of the growing interest in ERNs, from many different stakeholder groups, and considering the fact that participation in the Brussels workshop was of necessity limited to 60 experts, the fruitful discussions and the questions raised have been summarised here in the hope that they will contribute positively to the debates and activities surrounding ERNs. **Where relevant, information has been updated (in red font, accompanied by this symbol -!) and answers clarified to reflect conclusions from subsequent meetings and developments in recent months.** In particular:

- The advanced drafts of the PACE-ERN deliverables (the Assessment Manual and Toolkit) is available here -http://ec.europa.eu/health/ern/assessment/index_en.htm#fragment6
- The Reports and resources from the 2nd official ERN conference may be consulted here - http://ec.europa.eu/health/ern/events/ev_20151008_en.htm
- RD-ACTION has launched an informal 'Matchmaker' exercise to support collaboration and communication: see http://www.rd-action.eu/european-reference-networks-erns/
- RD-ACTION has launched a TaskForce on Interoperable data sharing in the framework of ERNs
- EURORDIS has published a set of FAQs specifically for patient advocates http://www.eurordis.org/sites/default/files/ERN%20Q%26A%20Final..pdf

Background – the Workshop

A workshop was organised by the EUCERD Joint Action within the scope of Work Package 8 ('Integration and Sustainability'), in conjunction with the European Commission (DG Santé). This was a large workshop, involving approximately 60 participants, which took place in Brussels on 1st and 2nd July 2015: the venue for Day 1 was the EC's Albert Borschette Building on Rue Froissart Brussels; on Day 2 the participants were hosted by the Brussels Office of the Instituto de Salud Carlos III (ISCIII), Rue du Trône.

The workshop was attended by 40 experts from clinical rare disease fields: 19 of the 21 Thematic Groups of Rare Diseases espoused by the Addendum to the EUCERD Recommendations on Rare Disease ERNs were

[1] Co-funded by the EU : contract 20112201 (DG SANCO)

[2] The policy-focused activities of the EJA evolved in Autumn 2015 into the Policy work package (6) of the new Joint Action for RD, RD-ACTION (677024).

[3] http://ec.europa.eu/health/ern/docs/faq_establishing_ern_en.pdf

represented, by experts with relevant clinical/networking expertise. In addition to this diverse group of clinicians and academics, 22 experts attended representing key stakeholders connected to the ERN topic, from DG Santé, the Joint Action, the Joint Research Centre, Rare Diseases Europe (EURORDIS), the European Observatory on Health systems and Policies, European Union of Medical Specialists (UEMS) and PACE-ERN (the consortium awarded the Tender to elaborate an Assessment Manual and Tool-Kit for ERNs).

Aim of the Workshop

The overall aim of the workshop was **to support the rare disease (RD) field in preparing for the first Call for ERNs, expected in early 2016.**

Specific Objective addressed:

The specific objective was to assess and attempt address the current 'readiness' for RD ERNs. The context for these discussions was established, in terms of the background to ERNs, the fundamental legal basis of ERNs, relevant RD policy documents, and examples of the first draft components of the tool-kit under preparation by the Tender generating the Assessment Manual for ERNs. Common questions relating to RD ERNs were raised and discussed, to provide maximum detail at this stage of the opportunities afforded the RD field through these new infrastructures.

The workshop began with a series of presentations, to establish the road to ERNs and the status quo, from various stakeholder perspectives.

EU Policy on Rare Diseases (Jaroslaw Waligora)

The Presentation highlighted European Commission activities and investment in the field of RD. The potential in this domain for added value via a collaborative, European-wide approach was demonstrated. The major policy documents were defined and key focal areas were summarized: the status quo of national plans and strategies for RD; definition, coding and inventorying; research; registries and databases; and patient empowerment. The nature and purpose of Joint Actions were explained, along with the role of the EUCERD and subsequent Commission Expert Group on Rare Diseases.

EUCERD Recommendations on Rare Disease ERNs (incl. 2015 Addendum) and Centres of Expertise (Kate Bushby)

This Presentation summarised the scope and purpose of the *EUCERD Recommendations on Quality Criteria for Centres of Expertise for RD* (2011) and of the *EUCERD Recommendations on RD ERNs* (2013). The rationale for an Addendum (2015) to the ERN recommendations was explained and the content summarised. Grouping rare diseases thematically is essential in order to create a logical and feasible number of RD ERNs which, collectively, can ensure that no RD is left 'without a home' under an ERN. Kate discussed some of the challenges inherent in grouping RD in this way, and clarified the realistic implications of the Addendum model for centres of expertise/healthcare providers wishing to lead or join ERNs in the RD field. Common misconceptions and confusions were highlighted (for instance, the distinction between the scope and function of a Centre of Expertise, on the one hand, and an ERN on the other, was made more explicit).

The Road to ERNs (Enrique Terol)

Enrique Terol presented the Policy and legal background to the concept of ERNs. He highlighted the major watershed moments in the timeline to-date (e.g. the publication of the Directive on the Application of Patients' rights in cross-border healthcare). He explained the overall vision for ERNs: ultimately, these will be networks to improve quality and safety and access to highly specialised healthcare in Europe. The Delegated and Implementing Acts were summarised, with particular emphasis on a) the criteria a network will need to fulfil to be designated an ERN and b) the criteria a healthcare provider will need to meet in order to be a member or an ERN. The role of Member States (MS) in the process was discussed. The Tenders working to deliver tools and resources for ERNs were also presented.

The Board of Member States of ERNs – roles and responsibilities (Till Voigtlaender)

The presentation summarized the role of the Board of Member States (BoMS) of ERNs in approving ERNs. The BoMS consists of representatives from across the EU Member States and European Economic Area (EEA). The Board's main roles and responsibilities[4] are to:

- Develop and maintain rules of procedure for the Board of Member States (functioning and decision-making process);
- Review the assessment reports and recommendations from the Independent Assessment Body (IAB);
- Give final approval of applications for ERNs;
- Approve proposals to add one or more members to an existing ERN;
- Approve the termination of an ERN; and
- Decide on the loss of membership of one or more members of an existing ERN.

Patient Involvement in RD ERNs – Yann le Cam (EURORDIS)

This presentation clarified the meaning of 'patient involvement' in the context of ERNs.[5] The term 'patient involvement' can be nuanced, to be very weak or very strong. The vision of ERNs, enshrined in law and in policy documents, is now very close to what patients expected and desired. The patient message has always been that a stepwise approach is key, and by this is meant stepwise in terms of disease coverage and also geographical coverage. The more clinicians and centres are engaged in quality discussions, the better for patients. **Patients also wish to see a dynamic development in terms of functions and services of ERNs: patients would insist that data collection and registries should be the number one priority, but after that the development of services and tools should be stepwise**. Once established, it is very important to the patient community that RD ERNs are not isolated but will be part of a new ecosystem of data and innovation. There have been considerable changes in recent years in terms of the flexibility in executing clinical trials, and increasing regulatory flexibility is foreseen in the future for RD, but only if there is real capacity to collect a critical mass of high quality data. This is the reason for demanding that registries and data collection are a priority of RD ERNs.

[4] **Update**- further details on the strategic role of the BoMS was published in January 2016 http://ec.europa.eu/health/ern/board_member_states/index_en.htm

[5] **Update-** further information aimed towards patient advocates was published in January 2016 - http://www.eurordis.org/sites/default/files/ERN%20Q%26A%20Final..pdf

Patient organisations anticipate that Horizon 2020 (H2020) could soon launch calls stipulating that when undertaking clinical research, EMA approval should first be obtained; similarly, it is feasible that in order to participate in certain future H2020 or IMI grants an institution might need to be part of an ERN. A key consideration for patients is how to participate most effectively in the Boards of each ERN. It would be impossible to have a representative for each disease under a network; therefore, EURORDIS has determined to create 'European Policy Action Groups' (EPAG) organised by RD groupings. These EPAGs will participate not only in ERN discussions but also in topics such as registries and data collection, research and therapy development, screening and genetic testing, etc. EURORDIS has already established an EPAG dedicated to rare cancers, with 8 elected members. A final key message from the patient perspective is that ERNs must not be viewed as administrative entities, entailing merely a greater burden for clinicians; rather, it is essential to focus on patient health outcomes and clinical excellence.

Assessment scheme and examples of ERN applications (Charles Bruneau)

The presentation[6] from the PACE-ERN consortium explained that the mapping element is now complete. They have explored various models and practices including FSMR in France, 'Green Corridors' in Lithuania which speeds up access to care, etc. The general findings from the mapping affirmed that patient involvement is key. Where existing national assessment programmes were identified, the themes used correlated well with the themes outlined in the Delegated Decision. Most importantly, the research to date has affirmed that all stakeholders appear to see the value in ERNs.

Discussion Sessions

Procedural questions on establishing ERNs

When will the Manual become available?	**The Assessment Manual and Tool-Kit is expected to be published in late 2015 or the beginning of 2016.** ! Advanced drafts of the Assessment Manual and Tool-Kit are now available: - http://ec.europa.eu/health/ern/assessment/index_en.htm#fragment6
Will ERNs be considered legal entities? What about Intellectual Property rights? In some projects, it was reported, IPR has been a limiting factor for success: when discussing multidisciplinarity and the exchange of information between counties, it is necessary	ERNs will likely be 'legal entities' only in the sense that they are licensed to use the ERN trademark. Concerning IPR, in principle the deliverables of the ERN will be public (i.e. deliverables such as Clinical Guidelines) and these will need to be shared. However, there is a need to discuss the IPR issue further as there will certainly be research in the network.

[6] **Update** - The Assessment Manual and Tool-Kit are now available as advanced drafts - http://ec.europa.eu/health/ern/assessment/index_en.htm#fragment6

to have a clear scenario on IP rights.	
What formalised structure will ERNs assume?	Health care provider applicants will be approved member of a European Reference Network which will have an institutional value. Networks' Members should be licensed to use the 'European Reference Network' logo. The logo, owned by the European Union, should constitute the visual identity of the Networks and their Members
Some the existing networks (formally or informally) involve many experts from outside of the EEA: how might these countries participate?	In addition to the EU 28, the three EEA countries are eligible and indeed already participate in the Board of MS for ERNs. The policy and legal documents state that members of ERNs are expected to collaborate with others centres and networks; however, it is not possible to include them as formal members as they cannot be included in any formal EU assessment of the centres and networks. Therefore, external participation and contributions are encouraged, but cannot formalised.
Participants highlighted the concern that MS have adopted radically different approaches to designating centres of expertise (CEs) for RD. Many MS do not yet have formal designation processes to acknowledge RD expertise. In some countries, many CEs are self-nominated and the formal designation process as such is still embryonic; therefore, how can they participate in ERNs?	Although a key pillar of the RD policy agenda has been to encourage MS to officially designate national providers as 'CEs for RD', in accordance with Council Recommendation (2009) C 151/02, this is in fact a separate issue to the national endorsement process for ERN participation. MS have full capacity and responsibility in the endorsement process for national centres to join ERNs: in some cases, countries will endorse officially designated/recognised CEs for RD, but in others this will not be the case.
If a MS has not yet decided how to endorse participation of national HCPs in ERNs, what are the options to participate in ERNs?	Each year there will be an opportunity to join an existing ERN, as stipulated in the Implementing Decision (although it was pointed out that this would preclude such centres/countries from coordination of ERNs, if a Network had already been approved in that area). It was suggested that meanwhile, HCPs could join as an Associated National Centre or a Collaborative National Centre, with nothing to prevent centres taking this route initially and then in future, once a formal national procedure *has* been established, applying for the full membership.

Participants sought further clarification around the concept of Associated National Centres and Collaborating National Centres (as mentioned in the Delegated Decision). The Board of MS and the CEGRD have also discussed this issue. It would be beneficial for smaller countries to understand the definition of an 'Associated' and a 'Collaborating' partner – and also beneficial for large countries, to appreciate how best to organise membership/affiliation of ERNs. The issue may be raised again with the Board of MS of ERNs; however, when the Acts were being elaborated, the MS opted to avoid any specific definition of these terms, on that grounds that they did not appear in the CBHC Directive. This may preclude any further specific criteria, therefore.[7]

Experts discussed how help and support might be provided, both during the application stage and once an ERN is operational. Some proposed a helpline could be set-up for coordinators. Alternatively, guidance could be sought from the representatives of the ERN Board of MS. Others suggested that the cross-border healthcare National Contact Points should be the first port of call; however, some participants were concerned that NCPs have varying levels of knowledge and expertise. Several workshop participants expressed concern that their MS was not yet prepared for ERNs, or else simply was not interested in participating.

In the absence of key tools such as the final Assessment Manual and the deliverables of the Services Tender, could the first call for ERNs not be deferred?	The first call must be launched within 2 years of the publication of the Acts; therefore**, May 2016 is the terminal point for the first Call.** It was pointed out that in such ambitious enterprises, there may never be a perfect state of preparedness, and it will take time for ERNs to reach full potential
Will there be subsequent calls for ERNs?	Probably. Participants reported that there will likely be more than one call, although this will need to be agreed with the MS, as will the interval between the 1st and 2nd calls. ! In January 2016 the Board of MS of ERNs published its ***ERN implementation strategies*** which states that "As it will not be feasible to establish all thematic networks at once due the different level of maturity of the current pilot projects and possible proposals, further calls should be launched in due time, as provided in the legal base, so that all potential thematic groups of rare and low prevalence and complex diseases and conditions would have the

[7] **Update -** In January 2016 the Board of MS published ***ERN implementation strategies*** – http://ec.europa.eu/health/ern/docs/ern_board_implementationstrategy_en.pdf which confirms that **"for the sake of inclusivity each ERN must indicate the entrance pathways for the affiliation of centres others than the approved members of the Network".** Although emphasising that "Evaluation of affiliated partners is a matter of national designation" the document confirms that "It will be very important that the BoMS agrees a minimum set of criteria that, used at national level, would give the affiliated partners a common ground and homogeneity".

	opportunity to be covered by a Network."
Will PACE-ERN define the disease-specific operational ('vertical' criteria of the Delegated Acts Annex II) for each ERN?	The Manual defines horizontal *operational* criteria which will apply to healthcare providers or networks in a *generic* way, with the overall goal of quality improvement, which demands a degree of oversight. Furthermore, there are a set of specific criteria and conditions that may vary depending on the scope of the concrete area of expertise where ***the proposed Network has to recommend and document its specific criteria on the required competence, experience and activity of all possible members of the ERN.*** Those criteria will be used to assess the level of fulfilment of each of the applicant healthcare providers.

It is important to always return to the basic model here. A network is made-up of centres and each network will have one coordinator. The identity of that coordinator needs to be decided as soon as possible. The coordinator and the member providers must discuss what the necessary level of expertise might be for their disease area, and how that expertise will be benchmarked. This information will form part of the ERN and member proposals. There is an obvious correlation here to maturity of existing networking structures: if a disease field is not yet in a position to agree such things, this will be problematic**.**

It was proposed that ERNs will require not only healthcare expertise but also research expertise, in fact basic as well as clinical. The importance of educating and integrating basic researchers was stressed. The panel confirmed that the Acts do not exclude basic research. The participation of pharma in this process will need to be clarified.

How might ERNs interact with the pharmaceutical industry?	This is not yet clear, but procedures will be defined as this is potentially a very important topic.

The group discussed how membership of an ERN might affect relationships between HCPs and their respective national health insurance companies. Some proposed that membership *may* facilitate the process of gaining approval from the payers to perform NGS for their patient, or to send a patient abroad for treatment.

How might membership of an ERN enhance relationships between centres and their national health insurance company?	It is important to remember that patients' rights to seek healthcare outside of the MS of residence are unchanged by the advent of ERNs: the CBHC Directive and the European

	Social Security Directive have established the legal rights and responsibilities.

Establishing ERNs by Thematic Groupings – the 2015 Addendum

The practicalities of organising existing partners and colleagues into an ERN were discussed at length; for instance, a participant from the rare skin disorder field pointed out that there are 400 Genodermatoses. The Epidermolysis Bullosa (EB) field specialises in one disease, and already has a network involving 70 partners in over 50 countries. If an ERN is proposed along these lines, will all of these 70 partners need to provide full details of their expertise and operations? Although the minimum number of HCPs for an ERN is 10, there is no maximum: it is possible to have 10 members or 15 members of 50. Remember though that a representative from each of those members would need to serve on the Board of that ERN (a governance board), so the EB filed would have to consider if 70 partners is desirable – if so, indeed all would need to submit membership applications. However, coordinators should think carefully about member HCPs and only seek membership from those which are most proficient and which will meet the criteria. And in fact, there is an extra layer of complexity to consider here, as an EB network would –according to the expert conceptualisation of RD ERNs- itself sit within a wider 'Rare skin disorders' ERN, encompassing more than just Genodermatoses. Therefore, the interrelation between these groups should be considered when conceiving the structure of this ERN.

If a network focuses primarily on paediatric care at present, is it acceptable to set-out a strategic plan to demonstrate how to expand into adult services?	Realistically a stepwise development will be necessary in some cases, in terms of disease coverage and geographical coverage (and presumably age too)

A representative from the rare GI field with a background in specialised anorectal surgery noted that surgical procedures and surgical themes do not appear prominently in the debate, nor in the CEGRD model. He testified to the benefits of sharing such expertise, however: patients suffering from these sorts of malformations are affected from birth and need to see very experienced paediatric surgeons at the right time to make a difference. Otherwise, the child will face lifelong problems which are of course costly to the health and social systems at large; therefore, there are major economic as well as health benefits to addressing these problems properly through highly-skilled surgical interventions. A representative from the rare urology field agreed that in certain cases there is a major benefit to patients actually travelling to receive highly-specialised surgical interventions – there is a significant need for training here.

Why are none of the Themes in the CEGRD model dedicated to specialised surgical procedures?	Specialised surgery and interventions are extremely valuable and valued, and are very much part of the 'frontline' of ERNs. However,

	such procedures are viewed as more cross-cutting. In terms of elaborating a proposal, the complexity will be to identify appropriate disease-oriented peers and submit the proposal together.

The participants discussed the concept of professional esteem and reputation, and how this could create challenges in establishing ERNs in line with the CEGRD model for RD: in some cases networks have been established and are working well, but nonetheless there are certain high-profile centres and clinics which refuse to cooperate and do not share their expertise. Some expressed concerns that such 'rival' factions may set-up ERNs in the same sort of disease area.

How might 'duplicate' proposals for ERNs in the same area be handled?	The process remains to be formalised here – but in all probability, if the EC receives two proposals addressing the same disease area, only one ERN would be approved. There will likely be a mechanism by which the proponents are asked to rethink and restructure themselves. But it is hoped that expert communities are open to working together and arrange to submit a single, joint proposal. ! In January 2016 the Board of MS of ERNs published its '***ERN implementation strategies'*** which confirms the importance of "limiting the number of networks to one per thematic group."
Will 'eminence' and reputation of HCPs be influential in selecting coordination and membership of ERNs?	Professional competitiveness will always exist - but in the case of ERN membership/leadership it will ultimately be a matter of 'evidence over eminence', as all centres will need to document their expertise.

The group discussed the challenges inherent in publishing outcomes of centres specialising in ultra-rare diseases. The fact that the number of cases will inevitably be very small may invite negative interpretations. One must be wary of how to interpret such figures for very rare conditions.

It was noted that the Addendum model for RD ERNs espouses a single network for both paediatric and adult patients (with the possible exception of the cancer field). Certain existing networks are very much geared to children: there *are* adult services of course, but in terms of organisation and

management it will be very difficult to incorporate adult services into existing specialised services in a timeframe of 6 months (i.e. time to the 1st call for ERNs). Therefore, clinicians questioned if it would be acceptable to have a 'business plan' to improve over time and gradually address the areas which are not 'ticked' at present.

If a network focuses primarily on paediatric care at present, is it acceptable to set-out a strategic plan to demonstrate how to expand into adult services?	Realistically a stepwise development will be necessary in some cases, in terms of disease coverage and geographical coverage (and presumably age too)
Would a HCP be able to join an ERN if it does not possess expertise in *all* the diseases that could fall under the thematic scope or grouping of a Network proposal?	Yes a HCP could join a proposed ERN even though it does not possess expertise in all diseases under the scope of the proposed network.

Broadening current network scope – challenges and opportunities

Many clinical experts currently participate in disease-specific networks (for instance 'pilot' networks, established by EC funding in the past) or in networks dedicated to a relatively small number of diseases: <u>is it possible for such networks to simply 'transform' into ERNs, retaining their current scope?</u>	No, this is not the aim of ERNs which represent a collaboration in healthcare. Many former networks were collaborating in different fields of research. The goals and criteria established in the legal acts are mandatory and therefore the current pilot networks or groups wishing to apply as a Network proposal shall assess themselves and reach an agreement on how to fulfil those criteria. It would be advisable for the current narrow disease-specific networks to expand their focus and increase their networking associations in order to better reach the goals and minimum number of participants.

Although most of the clinical experts present at the workshop currently participate in disease-specific networks, or networks dedicated to a small number of diseases, the key message is that these networking associations need to increase and the scope will need to expand beyond present collaborations. In the Neuromuscular field, for instance, it will not be sufficient to address only inherited muscle diseases, and 'new' affiliations with expert communities in acquired neuromuscular disorders, peripheral neuropathies, Amyotrophic Lateral Sclerosis etc. will need to be established. However, the expectation is that being part of a large ERN will bring added-value that fields lack at present; for instance, there will foreseeably be access to shared communication tools and superior e-health resources etc. But beyond this, it is hoped that sharing diagnostic tools etc. will demonstrate how smaller, functional networks can be enhanced by operating more broadly. It was explained that the Huntington's disease field, for instance, is already well-networked, with over 100 sites affiliated to the EHDN. They share tools, a registry etc.; therefore, participants questioned the concrete benefits to HD experts becoming part of a rare neurology ERN.

What do the more established expert communities have to gain from ERNs – why should they collaborate more broadly across disease areas when they have a successful network dedicated to one disease or a particular subset of diseases?	For ERNs to be successful and truly benefit patients and professionals all across Europe, reducing inequalities and spreading good practices, stakeholders need to think beyond their immediate 'boxes' and appreciate their role in the overall strategic vision. If an ERN *was* set-up solely for, say, one rare neurological disease, it might benefit those diseases and communities which are already 'strong'; however, in creating a 'gap' in terms of disease coverage (for which there is no existing 'plug') it would not help those diseases which do not have such well-developed tools and infrastructures. There is a duty here to consider the greater good, as well as the more specific benefits which will come from **greater networking and opportunities to access shared tools and resources**.

Although the group agreed there is a need to consider the 'bigger picture' and greater good here, many participants stressed that having more details about the concrete benefits (e.g. IT platform tools) they could expect by adapting to this overarching model of grouping RD into ERNs would make it easier to persuade colleagues and local authorities that ERNs are worth the perceived administrative burden.

The workshop participants discussed the status of the Groupings (as promoted by the CEGRD Addendum). Some questioned whether there was 'room for manoeuvre' at this stage. For instance, participants questioned the logic in 'merging' anaemias with coagulation disorders under a rare haematology ERN. The panel explained that the Addendum is not legally binding, and can be adapted if there is a strong justification. One could, in theory, make the case for separating disorders relating to platelets from those relating to red blood cells; however, this argument could apply to many disease areas and given that this Groupings list was agreed only in June 2015, and represents a long process of expert engagement and review, it is inadvisable to think already of reducing this list of Thematic ERNs. There will never be a perfect list that meets the expectations of every stakeholder.

It was pointed out that in seeking to break down the Groupings further, one should consider the needs of the smaller MS too – the more one separates out the diseases, the more the small countries will struggle. They probably will not have a CE for rare coagulation diseases and another for rare anaemia, and will have a better chance of having at least one Centre which could be viewed as possessing expertise in 'rare haematology'. Again, the panel stressed that **the CE level is a different granularity to the ERN level.** Not all healthcare providers in, for instance, a rare haematology ERN, will need to be expert in all rare haematological disorders, or even all types of haematological disorders. The advantage is that if you have a CE for von Willebrand's Disease, for example, which has limited knowledge of rare anaemias, it could join the rare haematology ERN and become more aware about *other* rare haematological disorders over time, and access this expertise more easily. Without this approach, one would omit a centre which has expertise in a single, specific haematological disease, which actually may have no network at present.

The networking communities which have achieved the most to-date should aim to see the bigger picture here: ERNs are not merely about networking the most robust communities, but rather ensuring coverage across all diseases.

One goal of networking is surely to help less well-developed centres raise their standards, those which may not be at same level of expertise as others: but how can this happen when realistically, only the best centres will meet the member criteria completely?	For ERNs to be successful, the first networks to be established will logically be those with robust collaborations already, those with experience in Networking between CEs. The goal must be achieved in a stepwise manner, with the 'stronger' areas supporting the others and showing the way. ! Acknowledging this dilemma, the Board of MS of ERNs published '***ERN implementation strategies'*** in January 2016 which confirms that "for the sake of inclusivity each ERN must indicate the entrance pathways for the affiliation of centres others than the approved members of the Network".

Several participants agree that the Groupings list should not be changed at this juncture. A strong recommendation is that one should consider the 21 Headings (e.g. Rare Metabolic Disorders) as **virtual bodies under which sub-groups sit.** When elaborating proposals and defining the structure of an ERN in any given disease area it is necessary to take account of what exists already, and to keep things as simple as possible; for instance, in the Addendum model the Rare Metabolic Diseases ERN would be coordinated overall by a single nominated person in a single healthcare provider. Therefore, the community will need to decide how to integrate existing networks and expert centres dedicated to *subsets* of metabolic diseases -such as lysosomal storage disorders, intoxication-type metabolic disorders, neurometabolic disorders etc.- under that single 'umbrella' Thematic heading.

Who decides which category diseases fall 'under'?	It will be for the healthcare providers and stakeholders preparing a Network proposal to decide on this very important issue, to argue and defend its proposed ERN scope. The recommendation issued by the Commission Expert Group on Rare Diseases on a model of grouping should give an orientation of what is intended, which means several disease entities are grouped together. Examining who takes care of a patient at present could be a useful way of approaching this. The specialists themselves are best-placed to determine which ERN grouping their disease falls under as this is something best addressed at the grass-roots level by those expert in the diseases. However, a mapping of some sort may be advisable during the ERN proposal preparation stage, in order to assess the sort of disease coverage desired by that ERN at the end of the strategic plan for ensuring comprehensive coverage of RD to address patient needs.

Is there formal guidance on how to integrate existing disease domains under a single overarching ERN heading?	No, there is no legal requirement to structure ERNs in this way. It is merely a practical suggestion to accommodate the existing clinical realities and substructures. The Board of MS of ERNs may offer guidance, perhaps. But there is nothing to prevent a coordinator 'mapping' out the sub-domains within each Thematic

	Grouping and negotiating a way to bring that expertise together via a Board, for instance. The governance is not restrictive here. Thus, the coordinator of a Rare Metabolic ERN, for instance, could define the sub-domains (such as LSDs) and even identify notable single-disease-networks (e.g. a Gaucher Disease network) within that, agreeing how to 'bring-in' all of the required expertise over time.
Some of the Groupings will encompass a large number of rare diseases – how can this be managed?	Although the lower limit for membership of an ERN is clearly defined in the Legal Acts (at least 10 health care providers in at least 8 MS) there is no formal 'upper limit' in the legislation; given the necessity for each member HCP to appoint a representative to the Board of that ERN, the workshop participants proposed that limiting the number of HCPs joining a single ERN in any given MS would facilitate effective governance, although how this might be achieved is uncertain.

Participants discussed the merits of a transversal genetics ERN. The panel questioned the wisdom of having horizontal networks on specialities such as proton beam therapy or genetics, as it becomes too removed from the care concept and from the rest of the ERNs. Some felt that a transversal genetics/genomics ERN would have particular benefits for undiagnosed patients (the possibilities offered by the H2020 call 'solving the unsolved' remain to be seen and, it was suggested, could be of relevance here). The panel confirmed that genomics and genetic testing will certainly play a key role in all Networks; however, genetic testing is not specifically mentioned in the Acts. Perhaps a parallel would be to state that although surgery will be a key part of many multidisciplinary teams in ERNs, 'surgery' will not be its own ERN. And similarly, genetics will be a key part of all ERNs but not its own separate network.

The 'Rare Cancers and Tumours' Approach

There was extensive discussion about how the rare cancer field might be planning to integrate with ERNs. One option would be to consider a three-way division, into retinal blastoma, hepatoblastoma and very rare solid tumours. It was noted that the paediatric oncology community is very well-established, which is an advantage here. The vast majority of paediatric cancers are classed as rare, and this has been the starting point for discussion in networks such as

EXPo-R-Net. Nonetheless, there is a huge degree of variation in terms of diseases, treatments etc. and the key question is 'what is the real need for patients'? It is very difficult to speak in terms of expertise and who is or is not an expert; therefore, the field created the term 'hubs of coordination' instead of 'Centre of Expertise'. One option for the rare cancer field might be to begin with three networks dedicated to paediatric cancer, haematological cancer, and solid tumours. The multidisciplinary needs are specialised and different for each, and the surgical worlds do not overlap at all. So the plan might be to adopt a step-wise approach and envisage sub-networks under the umbrella of a 'paediatric oncology ERN'. Other representatives of the rare cancer field expressed concern, on the grounds that one would need more ERNs to address the needs of the community, perhaps as many as twelve.

The IT Platform for ERNs

How will ERNs support the exchange of knowledge and expertise?	This is a complex issue, and the expectation is that as ERNs are implemented and developed, more elaborate means of exchanging expertise and knowledge will emerge. The IT platform is a key part of the ERN infrastructure and is expected to deliver on the mandate of the CBHD Directive that expertise should travel (as opposed to patients, wherever possible). eHealth tools will be important to enable virtual exchanges of knowledge and data.

Tele-medicine/e-health tools will be essential to the success of ERNs and the delivery of cross-border healthcare. Clinical boards and virtual tumour boards, for instance, are already recognised as essential to excellence in care for cancer and complex diseases. The EC will examine exemplary e-tools (e.g. tele-cardiology tools) which may be viewed as solutions to facilitate the delivery of cross-border care. If necessary, the EC will undertake a pilot to develop these e-health solutions. It seems that currently there is limited formal use of available telemedicine tools; however, this is not so much a technical issue but rather a problem of willingness and strategies on the part of healthcare managers. Therefore, it is necessary to determine what e-health tools are actually already out there (bearing in mind that many of these are open source and that new investment is not always necessary).

The EC will set-up a public website for ERNs, for everyone to access. Then there will be the Secure IT ERN platform which will, in time, comprise of the following:

- a shared Intranet for all ERNs, which will store all the open, common tools and will have a step by step development based on your needs. We will include basic elements here

- a specific intranet for each ERN.

The platform will enable the exchange of clinical information and patient data via databases/data-sharing and patient registries. The platform will have dedicated communication and conferencing tools. It will support the exchange of images, X-rays, diagnostic tests, pictures (PACS) etc. Then there are building blocks e.g. 'Clinical decision-making tools' meaning not only clinical guidelines, but also other kinds of tools such as algorithms, a document repository and collaborative tools (e-rooms etc.). Other IT tools will be related to the capacity of the network in question e.g. there will be building blocks related to particular disease areas –there are solutions already for exchanging

images etc., so options exist. But the key point is that the platform will come complete with technical support, which will be ongoing and web-based.

One of the most interesting and important benefits of the ERN approach will be increased opportunities and tools to share RD data across borders and deliver virtual one-to-one patient care within a network setting. There is a much broader potential, however, to share data (re-use data) for research, for healthcare planning, etc. Participants asked what are the expected opportunities for ERNs to actually share data?	**Various key questions remain unanswered, certainly in terms of broad data sharing across the ERN framework e.g.:** *The vision of the EUCERD Recommendations was that ERNs will use shared disease-related registries, with each member HCP contributing data to a shared resource (or making locally-held data query-able). Presuming that such registries will not be built from scratch, how will ERNs integrate and link existing disease-specific registries and databases to pool core data (which is essential in rare diseases)?* *Will RD ERNs have a common dataset/patient summary for cases discussed within the network? And/or will each of the 21 Themes define core disease-specific datasets?* *Will there be any link to the data contained within existing National RD registries?* *Will there be any exchange of data between ERNs and the JRC EU Platform for RD Registration?* *Will data be submitted to the RD-Connect Platform to support RD research and innovation?* **! In November 2015, RD-ACTION established a small *TaskForce on Interoperable data sharing in the framework of ERNs* to try to address some of these issues, in collaboration with the relevant stakeholders**
Data protection issues are crucial to the success of ERNs - have these been taken into consideration?	The EC is working on this, examining the legal agreements at work in existing pilots. However, the Delegated and Implementing acts clearly decree the sharing of data.

The possibility that the EC might contract with Adobe Connect to provide the common communication tools (for Coordinators and members to interact) was met with some concern. Several participants cautioned that if this is the solution that is being favoured by the EC, Adobe Connect will need to improve significantly (the frequent updates quickly lead to subsequent versions becoming obsolete and incompatible). Others pointed out that although Adobe is feasible to use in universities, it will not be accessible by institutions within the national health systems of countries such as the UK. Participants stressed that hospitals will insist on appropriate consents for sharing data. It was proposed that the Assessment Manual will clarify that informed consent will be necessary on various different levels/in different capacities: a model of informed consent will be provided.

In selecting a feasible tool to enable eHealth in a networking environment, the group discussed the fact that various 'assets' have been developed in the e-health field, and outside the health field altogether, which will support the transfer and sharing of data e.g. assets designed for patient pseudonymization via a unique e-Health identifier. Such assets are already being trialled in certain use cases but further work is needed.

Services of ERNs

Participants of the Brussels workshop brainstormed together to discuss what might be the real added value of ERNs. Suggestions included increased access for patients to gold-standard healthcare; a critical mass of high quality data; improved knowledge transfer; future potential for funding; clinical trial support. It is difficult for some to really 'drill-down' to identify the added value of ERNs, given the absence of the Assessment Manual and the results of the Services Tender. It was also emphasised that because the ERN concept is so new – it is the first pan-European enterprise of its kind in the health sphere- this is essentially 'uncharted territory' and therefore difficult to know what to expect.

Enrique Terol delivered a Presentation on 'The Expected Services of ERNs'

What Services will ERNs provide?	The Delegated Decision defines services to be offered by ERNs; however, a dedicated study on services is currently the subject of a Tender. **! This Tender has since been awarded to PricewaterhouseCoopers**

From the Delegated Decision, the core Services of an ERN could be identified as:

- Virtual consultations/telemedicine;
- Training and education

- Generation of clinical care guidelines
- Research

Enrique defined what is meant by 'healthcare services' in the ERN context: the term encompasses all services dealing with the diagnosis and treatment of disease, or the promotion, maintenance and restoration of health. 'Service provision' refers to the way in which inputs such as money, staff, equipment and drugs are combined to enable the delivery of health interventions (outputs). The meaning of the terms 'highly specialised' and 'complex' were also explained in the CBHC Directive context. Clinical services can be provided directly or from a distance. Non-clinical services primarily concern knowledge promotion, training, and research. ERNs will enable new ways to provide services to patients, both in terms of 'traditional' clinical services provided in a new forum and also innovative virtual services. However, cross-border clinical services delivered via e-health, for instance, raise challenges in view of the current lack of regulation regarding entitlements and tariffs. The EC is awarding a Tender to Services[8] to be provided by ERNs and their members. This will provide a conceptual framework for ERNs, explore the types of services to be offered, and establish the cost drivers of such services.

The Tender will establish a set of realistic case studies of patients seeking treatment or consultation within a network. The patient case studies will be based on thematic disease groups (e.g. metabolic) and also intervention area (e.g. specialised surgery, gene therapy, or radiotherapy). The Tender will then identify:

- the relevant networking activities associated with these patient case studies;
- the resources (human, material, structural etc.) and cost categories (staff cost, overheads, soft/hardware etc.) these activities entail
- the cost drivers of the activities and resources

The successful contractor will be contacting national authorities/institutions of highly specialised providers on the one hand, and highly specialised networking experts on the other, to explore their experiences. The outputs of this Services Tender will be:

1. A catalogue of healthcare services to be provided by ERNs and associated activities
2. Cost drivers and cost estimation of these healthcare services linked to ERNs
3. A method for cost estimation

[8] http://ec.europa.eu/chafea/health/tender-25-2014_en.html

Discussion on the Services Tender

Participants sought further details on how this Services Tender will estimate the cost of services associated with future ERNs. Several questioned the likelihood that their institutions would provide information for instance on resources and cost drivers down to such a level of detail as 'square footage of the centre' etc. Others doubted that colleagues in their centres would voluntarily reveal information on DRG (Diagnosis-Related-Group) interventions. And that in any case, such data is difficult to compare as every health system calculates the cost of such interventions in a different way. Colleagues raised the prospect that healthcare providers in an ERN may struggle to receive payment for services provided – it was reported that when conducting a survey for the EJA on cross-border testing of RD, some laboratories testified that payment never materialises.

The EC confirmed that the Tender contractor will not dictate these exhaustive services to be provided by ERNs: rather, the team will work with the expert community to ascertain which particular services are necessary in which clinical cases, and how they should be addressed.

Financial Support for ERNs and the costs of networking

The group stressed the need for financial support for the coordination of an ERN. Those with experience of leading networks affirmed that the coordination work is effectively the 'glue' holding a network together -and in this case, the role will involve coordinating at least 10 HCPs in at least 8 different MS- and must be supported financially. It was agreed that it would be useful to have some reliable estimates of coordination costs from existing networks, which involves identifying the cost drivers. A few participants shared their experiences here: the ESDN (European Skeletal Dysplasias Network) attempted to estimate the costs of e-health in action, by monitoring the number of people logging into teleconsultation calls etc, which provided some metrics. Paediatric oncologists estimated that the virtual tumour boards currently entail 10-12 e-consultations per week. What are the actual costs of assembling a multidisciplinary team? Dyscerne attempted to calculate the cost per case (in terms of doctors, coordination etc).

It was suggested that if ERNs indeed improve healthcare pathways and increase the accessibility of high quality healthcare, the costs associated with networking experts via teleconsultations etc. will be recouped through greater overall cost-effectiveness in healthcare systems. The EC confirmed it is exploring mechanisms for providing coordination support, although perhaps not at the administrative level.

What funding is available for ERNs?	The EC can only fund things when there is a mandate. There is no legal basis for funding an ERN, no mandate. The 3rd Public Health programme is funding the application and assessment process for ERNs and money will be set-aside to develop the IT platform, which will be offered free of charge to all ERNs. There may be options for funding through less

	direct sources e.g. ERNs could receive investment from the CEF (the Connecting Europe Facility), to adapt tailored telemedicine tools, for instance. Some MS may have the option to seek funding via European Structural Investment Funds
Will there be financial support for coordinating ERNs?	The EC is exploring options to provide so-called 'glue-money' to support the costs of coordinating these networks

It was reported that clinical colleagues are frequently cautioning the participants that they will not be able to conduct these sorts of networking activities on a voluntary basis, and again, the demands of coordination in particular were raised. Although expert RD clinicians wish to be involved, they do not have the time to complete burdensome administrative forms, and require appropriate support.

Various theoretical scenarios were discussed in the Brussels workshop, to try to imagine how ERNs will operate; for instance, if one envisages a European group agreeing on the best possible treatment for a rare cancer patient, via a virtual tumour board perhaps, and the consensus is that this patient needs to travel to a different MS for treatment, this is essentially the ERN treating a patient. Would the EC consider that any such services provided via the ERN framework itself are the basis for reimbursement? Participants noted there is still considerable uncertainty and concern over how to measure the costs of a service and apply these to ERNs; however, several emphasised that a workable system must be agreed, or else there is a risk that healthcare providers will simply state that 'service/procedure X or Y will cost a ballpark figure of, for instance, 1 million euros to perform.' It was clarified that the Services Tender will identify cost *Drivers*, not costs per se.

Participants were reminded that when it comes to a cost model for ERNs, what is needed is an estimation of the 'added' networking activities. If a patient visits a centre in his/her MS of residence for care, the payment will be processed as usual. If that patient cannot be treated locally and needs to be transferred to another centre in the ERN *outside* that country of residence, there are already, in the pre-ERN era, two means of enabling this: the Cross-Border Healthcare Directive and the Social Security regulation. This will continue to be the case, and is *theoretically* straightforward, as the process for payment/reimbursement of the centre providing that treatment is well-defined. The third –and more innovative- option would involve virtual provision of expertise amongst members of the ERN, for instance through a virtual tumour board: this virtual service provision is the shadowy part of the ERN model, at present, as currently there is no defined cost model for these virtual, e-health referrals. The group reflected on the fact that the EU has funded pilot networks in the past, and therefore it is not necessary to begin these discussions and cost estimations from scratch. The Services Tender should consult individuals already

coordinating networks in the highly specialised healthcare domain, to establish how services have been provided to-date and what the costs of virtual consultations etc. amount to in reality. This sort of data can be presented to MS, albeit with the important caveat that ERNs are something *new*.

An important issue when discussing the cost model for ERN services is the way in which a MS defines its 'basket of benefits'. Countries which operate on a social security system will have a specific code in order to receive specific named services and treatments. But if you are operating in a national health system, this process will not be so straightforward. The level of detail in any one country's 'basket of benefits' will have a significant impact for the patient; for instance, if 'care for Huntington's Disease' is included in the national 'offer', this could imply a full, cutting-edge neurological examination, or merely treatment of symptoms related with HD. But a rare neurology ERN will need to be able to assess what the services available to a particular patient with HD *should* be. That is the goal for the future.

Till reminded the group that calculating costs of treatment in national systems is a national issue and therefore is fixed. Instead, a more achievable and pertinent goal would be to identify the baseline cost of networking in a network involving for instance 10 centres (i.e. meeting the minimum criteria for establishment of an ERN), and in a network of 50 centres, etc. This will constitute an identified cost driver which one could present to the Commission.

Several participants affirmed that their networks have already attempted to estimate service costs in this way, which could be relevant for the Tender. For instance, the European Skeletal Dysplasias Network set-up a panel of ten experts to comment on particular cases: this has provided them with metrics to show how many reviewees log on per session (the next step would be to measure the length of time reviewers remain on the consultation). The RareCarenet team has also attempted to collect data on the costs of virtual cancer services. In paediatric oncology, similar metrics exist relating to virtual tumour boards. These virtual tumour boards are often also asked to provide diagnostic tests, however, which is a very expensive service. Till pointed out that nonetheless, if one provides the test there are ways to secure payment via the existing Directives: the process is often far from smooth, but there are options. What is missing, however, is a means of recouping the costs of that virtual consultation itself.

Benefits of ERN approach to Member States

It was agreed that an important step in persuading MS to properly fund such networking activities and services would be to demonstrate their associated cost **efficiency,** on the basis that investment in virtual consultation boards etc. will avoid future expenses resulting from, for instance, substandard/inappropriate surgical interventions or further tests. **Thus the healthcare system as a whole will actually benefit financially from proper investment at the appropriate point of the pathway. This could be an important means of demonstrating the benefits of ERNs for those MS which may question what they have to gain from ERNs** (for instance some MS may

consider their specialised healthcare provision to be exemplary already.) It will take time to demonstrate the economic benefit of ERNs (and in fact, this is more an issue for HTA bodies). In terms of persuading Competent National Authorities and hospital Trusts to participate in ERNs, clinicians should be emphasising the huge benefits of sharing knowledge and expertise, which will benefit patients enormously. The overall message here is that different MS need to promote the benefits of ERNs in different ways, depending upon national needs and priorities.

Notwithstanding the healthcare opportunities, participants believe that for some MS, the most attractive benefit of ERNs is the increased potential for RD data sharing and greater research opportunities. There is significant potential for ERNs to support research and attract interest from the pharmaceutical industry. Perhaps some future research funding streams will likely demand (or at least favour) participation in ERNs. It was pointed out that in countries such as the UK, which has strong links to US pharma, the language barrier with some of the EU MS can be a hindrance to clinical research; therefore, if ERNs can facilitate trials and regulatory compliance, UK stakeholders would likely view this as a major benefit.

Kate Bushby highlighted an important ERN activity not covered by national health system funding: participation in international registries. Some centres may be funded to contribute data to national registries, but participants argued that there should be a funding stream attached to international registries too.

Preparing for the Call

It was pointed out that this process of preparing for ERN applications would be much easier if the Tenders had already delivered their outputs – the planning here is not ideal. Several participants felt the timelines here are too short and are not adequate to bring together the people and the proposals required.

Can the first Call for ERNs be deferred?	The first Call for ERNs must be published within two years of the legal acts, meaning the end of May 2016 is the terminal point.

Therefore, realistically the best approach for the first call might be to concentrate on the 'low-hanging fruit', and allow other disease areas to organise themselves with more time. A stepwise progression is the most logical approach; however, it is imperative that the end goalposts are set at a high level. Several experts cautioned that not every ERN will be able to do everything from the beginning – Europe should not be too ambitious at first, but should be progressive. Therefore, there is a need to agree what are the immediate needs and priorities (the non-negotiables), then determine what is 'desirable'/optional, i.e. what can come later. Some concerns were raised about the Independent Assessment process: the experts agreed that these assessments must be uniform, and those performing the role ought to understand the networking environment and the

RD field. Yann proposed making use of the expertise in the Brussels group to support the Services Tender by identifying a few core current networking activities which should be possible to embed in all ERNs – thus beginning with a 'grass-roots' approach. EURORDIS' perspective has always been that a step-wise approach is best, in all aspects of ERN operations. In practice, what this means is that each ERN should limit the number of diseases addressed initially by its respective 'sub-networks'; however, it should demonstrate concrete plans to expand over time. Otherwise, if networks are pressured into making unrealistically ambitious proposals, addressing every possible disease under any given Thematic Group straight away, problems will occur when the first evaluation takes place five years hence

What could prospective ERN coordinators be doing at this stage, to prepare for the Call?	Colleagues should study the model for grouping RD defined in the Addendum, and consider the most appropriate ERN for their particular expertise. Then it will be necessary to visualise how the ERN might look (i.e. which 'sub-networks' or sub-disciplines should sit beneath that umbrella heading) and how comprehensive in scope it could realistically be by 2016, whilst outlining how the ERN should evolve further down the line/what is the ultimate vision (i.e. the step-wise approach). Coordinators and would-be members are encouraged to discuss their plans with respective National representatives on the Board of MS of ERNs, if they have not already done so. And at this point in time, clinical leads wishing to submit a proposal together must begin in earnest to discuss the appropriate thematic criteria for their ERN. They need to consider the appropriate healthcare pathways and models of functioning for the network. **! As of December 2015, interested parties should share their plans and collaborate by means of the RD-ACTION matchmaking tool for ERNs -** http://www.rd-action.eu/european-reference-networks-erns/

ANNEX 1 – Workshop Agenda

EUCERD Joint Action and European Commission Workshop

Realising Rare Disease European Reference Networks: a preparatory workshop for the Rare Disease field

Aim of the Workshop:
The overall aim of the workshop is **to support the rare disease (RD) field in preparing for the first Call for ERNs, expected to be announced in December 2015 or early 2016.**

Objectives:
The specific objective is to assess and address the current 'readiness' for RD ERNs. The context for these discussions will be provided, in terms of the background to ERNs, the fundamental legal basis of ERNs, relevant RD policy documents, and examples of the first draft components of the tool-kit under preparation by the European Commission (EC) Tender generating the Assessment Manual for ERNs. Common questions relating to RD ERNs will be explored and discussed, to provide maximum detail at this stage on the opportunities afforded the RD field through these constructs. Finally, the state of the art of networking practices across the various RD or low prevalence and complex clinical 'areas' will be shared and explored, with an emphasis on ensuring the best possible applications from the RD field result from the December 2015 Call for ERNs.

Day 1: Wednesday 1st July 2015

Venue: Albert Borschette Building on Rue Froissart 36, 1040 Brussels (room AB/2B)

11:00: Welcome to the Workshop (Kate Bushby and Enrique Terol)

Session 1- Rare Disease ERNs – where are we?

11:20: EU policy on rare diseases (Jaroslaw Waligora)

11:40: EUCERD Recommendations on Rare Disease ERNs (incl. 2015 Addendum) and Centres of Expertise (Kate Bushby)

12:00: The road to ERNs (E. Terol)

12:20: Board of Member States on ERNs – roles and responsibilities (Till Voigtlander)

12:40: Timelines for ERNs (Enrique Terol)

*13:00 – 14:00 – Lunch**

14:00 Patient Involvement in RD ERNs – Yann le Cam (EURORDIS)

14:30 Assessment scheme and examples of ERN applications (Charles Bruneau)

15:15 Discussion Session and FAQs (topics to include):

- Procedural aspects of establishing ERNs
- Distinction between Healthcare Providers and ERNs
- Organising ERNs by disease area and respective specialisms
- Financial Considerations
- What will be the added value of ERNs

(Moderators: Enrique Terol, Kate Bushby, Jaroslaw Waligora, Yann le Cam, Till Voigtlander, Charles Bruneau)

16:00- 16:30 Coffee Break

16:30 Discussion and FAQs cont.

18:00 Day 1 Ends

Day 2: Thursday 2nd July 2015

Venue: Brussels Office of the Instituto de Salud Carlos III (ISCIII), Rue du Trône, 62 - 1050 (Room III on 7th Floor)

09:30 Recap of Day 1 – K. Bushby

Session 2: Services for RD ERNs: where do we want to be?

09:50 Tools and services: European Commission IT Platform for ERNs and anticipated Services of RD ERNs (Enrique Terol)

10:10 Identifying good practices/successful services in existing networks, and how to go further: *providing healthcare in a network (and virtual) environment*

11:00-11:20 Coffee Break

11:20 Identifying good practices/successful services in existing networks, and how to go further: *supporting clinical guideline development*

12:20 Identifying good practices/successful services in existing networks, and how to go further: *training within a network environment*

13:00-14:00 Lunch (please note that this will be provided for participants in the restaurant downstairs)

14:00 Identifying good practices/successful services in existing networks, and how to go further: *facilitating an environment for clinical research and data-sharing*

Discussion: **Session 3 – Next steps**

15:00 Summary and Next Steps: what needs to be done between now and October? How might disease groups collaborate and organise themselves into broader fields? How should the report and outputs of this workshop be disseminated?

16:00 Workshop Closes