RD-ACTION Pre-Workshop Meeting between the Board of Member States of ERNs and the Applicant Network Coordinators

Wednesday 28th September 9–11 am

Venue: Thon Hotel EU, Rue de la Loi 75, B-1040 Brussels

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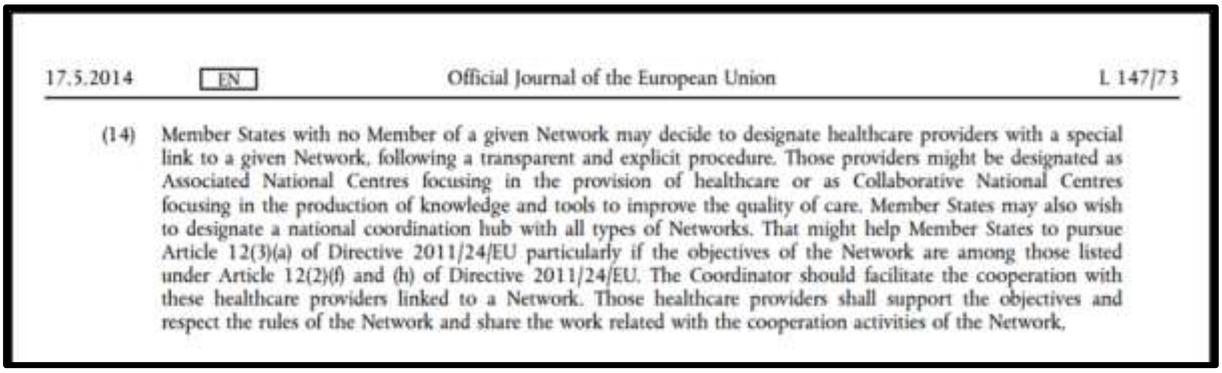
Introduction

Victoria Hedley, the Thematic Coordinator for the RD-ACTION Policy work-package (WP6) welcomed the participants to the meeting. The meeting has been organised by RD-ACTION ahead of the workshop on 'sharing data for virtual care in the ERN framework' and is intended as an opportunity for Board of Member States (BoMS) representatives to discuss key issues face-to-face with the prospective Coordinators. The meeting was arranged to leverage the fact that many ERN actors were in Brussels this week and the hope was that both Applicant Network Coordinators (ANCs) and Board Members would gain greater awareness of the challenges facing each of their stakeholder groups, and better understand each other's roles in the ERN implementation process. This was intended as the first in a series of meetings between the ANCs and the BoMS Representatives to strengthen relationships and develop a shared vision of ERNs. Although there are many issues of pressing importance relating to ERNs, two major discussion topics were selected to focus the debate.

Topic 1: Membership coverage and 'affiliation' to ERNs

(Discussion Chairs A. Damigou and L. Sangiorgi) Aikaterini Damigou, as chair of the Working Group on Affiliated Partners within the BoMS, summarised the Board's stance and future plans pertaining to this topic. Aikaterini explained that **'affiliation' with an ERN is defined in the legal base, both (14) of the Delegated Decision and (7) of the Implementing Decision:**



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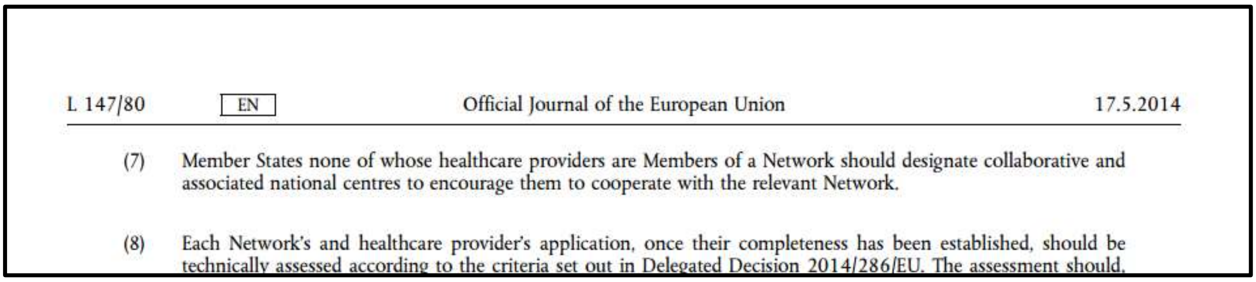
(14) Member States with no Member of a given Network may decide to designate healthcare providers with a special link to a given Network, following a transparent and explicit procedure. Those providers might be designated as Associated National Centres focusing in the provision of healthcare or as Collaborative National Centres focusing in the production of knowledge and tools to improve the quality of care. Member States may also wish to designate a national coordination hub with all types of Networks. That might help Member States to pursue Article 12(3)(a) of Directive 2011/24/EU particularly if the objectives of the Network are among those listed under Article 12(2)(f) and (h) of Directive 2011/24/EU. The Coordinator should facilitate the cooperation with these healthcare providers linked to a Network. Those healthcare providers shall support the objectives and respect the rules of the Network and share the work related with the cooperation activities of the Network.



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(7) Member States none of whose healthcare providers are Members of a Network should designate collaborative and associated national centres to encourage them to cooperate with the relevant Network.

(8) Each Network's and healthcare provider's application, once their completeness has been established, should be technically assessed according to the criteria set out in Delegated Decision 2014/286/EU. The assessment should,

Therefore, Member States have the option of designating three types[1] of 'affiliated' partner:

- Associated National Centre
- Collaborative National Centre
- National coordination Hub

Aikaterini emphasised that this **designation is a competence of each MS; however, in order to have some coherence it is necessary for the MS to work together to define the requirements and the process, hence the WG.**

Luca Sangiorgi (as a representative of the ANCs) highlighted several key issues from the perspective of the potential coordinators of the potential networks. The coordination task has been very demanding and time-consuming thus far – assembling (often vast) networks of HCPs, completing the forms and generating ERN documents has been challenging. This group of ANCs is very committed and dedicated. There are some common concerns, however, around the issues of future membership and affiliation. A particular concern for some of the ANCs is that HCPs with which they were planning to work in the ERN received national endorsement but this came so late in the process that they were unable to complete the application forms and assemble the requisite documentation. How can they join the Networks? When will the second call for membership come?[2] Besides these particular HCPs, it is difficult to see how the Networks will be able to accept new member HCPs for the first couple of years, as it will take time for the ERNs to establish themselves. Luca also emphasised the fact that although ANCs will -ideally- have already forged good relationships with their own National representative on the BoMS, **it would be beneficial to develop links between all BoMS members and all ANCs**, as each person's network will eventually have members and affiliated partners in each country.

Discussion – Other ANCs echoed Luca's concerns that the national endorsement for some very good HCPs -which would meet the specific criteria applied for their Network- came too late and the centres were unable to submit. In other MS, HCPs were not endorsed for bureaucratic reasons. Others emphasised that even without this endorsement, they will continue to collaborate with such centres, as in many cases there are years of history and good relationships there. A BoMS representative raised concerns about the prospects of managing very large Networks, emphasising that **the goal is not to include *every* HCP in Europe that has expertise in your disease area, but**

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[1] RD-ACTION postscript comment: The text of the Delegated Decision does not appear to preclude MS from designating more than one type of 'affiliated' partner: for instance, conceivably a smaller country may designate a centre with particular medical expertise in, for instance, rare hepatic diseases (although not reaching the mandatory criteria for full HCP membership) as an Associated National Centre to the 'Rare Hepatic' network, but may also choose to designate a leading hospital as a national coordination hub, to integrate with all ERNs.

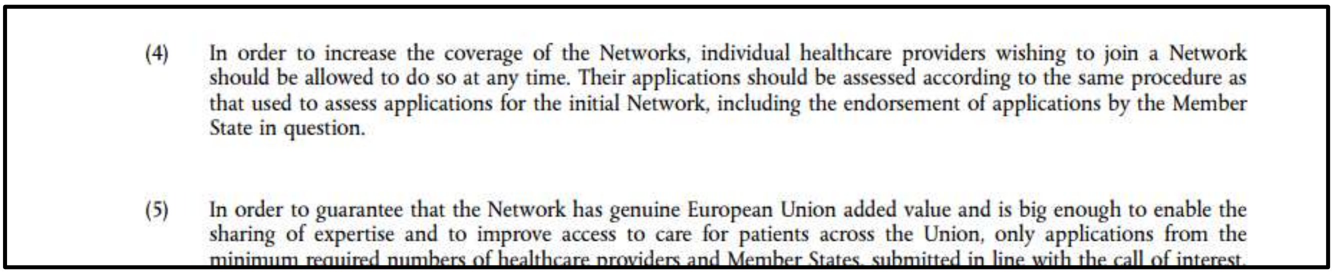
[2] As below (page 3) there will be no second call for membership *per se*.

rather to link a limited number of these via the formal infrastructure of the ERN. An RD-ACTION colleague added however that it was never the aim of ERNs to threaten or dismantle the strong relationships already existing in the field of specialised healthcare, and that of course ANCs should of course continue to foster collaborative relationships with centres across the EU and beyond.

Regarding delayed or overly bureaucratic endorsement procedures in some MS, another BoMS representative explained that national endorsement is not a matter of clinical expertise only but a combination of expertise on the one hand (which might be beyond dispute in a number or perhaps many instances) and the relevance for and integration to the long-term planning of healthcare structures on the other. In this context, it is important to realize that some countries with a long tradition in RD policy are more advanced in defining their expert HCPs than other MS and that ‘delayed’ endorsement may thus reflect the fact that these MS try to interlink the European and the national dimension of HCPs as Centres of Expertise to eventually guarantee a close link to the national healthcare system, to patient pathways, and to some sort of sustainability for these specific centres and their highly specialised activities. These procedures take time but highlight the fact that MS, although endorsement and participation of national HCPs in ERNs is voluntary, are willing to assume responsibility in selecting and thereafter supporting HCPs to take part in ERNs, which is eventually also in the interest of the whole ERN concept and thus also ANCs.

The participants attempted to clarify several issues (as confusions clearly remain around the issues of future calls, membership, and ‘affiliation’.) **When speaking of ‘future calls’ it is important to be clear on the type of call:**

- In terms of a future call for ERNs themselves, there is no information on this at present. A decision on a second call[3] was announced by the BoMS in its ERN Implementation Strategies document; however, this was before it was realised that proposals would be submitted for almost all thematic groupings proposed by the Commission Expert Group on Rare Diseases (in fact more TGs than the Addendum anticipated). Therefore, whilst the 24 proposals are being assessed, there is no information on a future call for ERNs themselves[4].
- Regarding the addition of new members to ERNs which receive approval in December; here, the legal acts are clear - there is supposed to be an open procedure for inclusion of new members (Implementing Decision (4), below). The EC confirmed that the criteria and the assessment process for these new members will be exactly the same as for the current applicants. But there cannot be blanket exclusions, as per the legislation (Implementing Decision (4)).



(4) In order to increase the coverage of the Networks, individual healthcare providers wishing to join a Network should be allowed to do so at any time. Their applications should be assessed according to the same procedure as that used to assess applications for the initial Network, including the endorsement of applications by the Member State in question.

(5) In order to guarantee that the Network has genuine European Union added value and is big enough to enable the sharing of expertise and to improve access to care for patients across the Union, only applications from the minimum required numbers of healthcare providers and Member States, submitted in line with the call of interest,

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[3] If one considers wave 1 (deadline 21st June) and wave 2 (deadline 21st July) of the 2016 call to be two halves of the <u>first</u> call

[4] Presumably the need or otherwise for a second call will depend upon various factors (for instance the success of the current proposals and the existence of any additional groups interested in establishing an ERN which did not have sufficient maturity in 2016)

The issue of 'affiliation' is something else entirely. The BoMS representatives emphasised again that the MS alone can designate the affiliated partners. As some of the ANCs raised the prospects that they will have several 'affiliated' partners in a single country, the RD-ACTION colleagues sought to clarify the purpose of 'affiliation': **'affiliation', whichever of the three possible modes a MS opts for, is a way to enable countries without a member in any given Network to have a 'gateway' or 'hub' through which to connect with the expertise of that ERN**. In practice, according to the Delegated Decision, this means that if country A already has a member HCP in ERN X, it should not designate *any* partners as 'affiliated' to that Network. However, if Country A has NO member HCP in ERN X, this would be when it invokes the affiliation route. [5]

All agreed that inclusivity and flexibility is key in the ERN concept. The 'affiliated' partner concept is a crucial piece of the puzzle – without these, how will the patient pathways work, how will patients be 'referred' to the shared expertise of the ERN? The decisions on which 'affiliated' partner to select in each country, and which type of affiliation to use, are very important and MS should carry out this task with a good view of the expertise in their country. The BoMS was asked whether it would be possible to produce a statement setting out the principles that underpin the extension of ERNs across all MS, ensuring a balanced network linked to each national network/framework, to support the ANCs here, as the latter currently receive many requests and are under great pressure to respond to centres expressing interest in their potential Network, without any solid information. There is little understanding in the wider field that the decisions are out of the hands of the ANCs themselves and a clear message from the Board could help to alleviate tensions. MS were also requested to be as transparent as possible in their decisions to endorse an HCP or not, to help stakeholders understand *why* such decisions are made. For MS, on the other hand, it would be very important to know the ANCs' opinions on how long it will take until their respective ERNs are fully operational enabling the networks to integrate further members and, in particular, affiliated partners.

Topic 2. Integration of ERNs with national health systems (Discussion Chairs T. Voigtländer and M. Scarpa)

Till Voigtländer emphasised the **importance of ERNs becoming properly embedded in national health systems.** ERNs are not time-limited projects, and must not sit in isolation: they are an innovation in highly specialised healthcare and have the potential for a meaningful improvement of patient care across Europe over time. For this to happen, ERNs must not be outside the health

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[5] RD-ACTION Postscript comment: Presumably in this case the country has two options at present – the route it takes should logically depend on whether:

a) the MS believes that it has an HCP which could, in future, become a member (i.e. a centre fulfils all criteria, and either received national endorsement too late, or else did not receive national endorsement at all in 2016 but is expected to do so in the future, once the MS is more prepared); or
b) it is clear that due for instance to the size of the country or the lack of expertise in this specific area, the MS has no 'candidate' HCPs in mind which would fulfil the membership criteria

The MS thus will presumably need to make a decision here, based upon their understanding of the national situation and capacities:
If a), the MS might choose to wait for full membership, if this seems fairly imminent; otherwise, if the country expects the full national endorsement process to take a substantial period of time (e.g. due to resource constraints/bureaucratic reasons), it may seem preferable to designate a centre as an 'affiliated' partner' with the expectation to seek full membership eventually but enjoy a link to the ERN in the meantime. If b) it would be logical to consider which centres/partners/institutions the country has which could be designated as the affiliated partner for that country in an ERN for rare renal, or for rare cardiovascular etc. In so doing, MS should also take into account existing national networks where one member might serve as affiliated partner and connection to the ERN for the whole network, if applicable.

system; instead, one should consider ERNs as a supportive part of national healthcare systems and ways need to be explored in all MS -taking into account the individual requirements in each country- to accomplish effective connection between the European level and national networks/frameworks, enabling integrated access care pathways for patients. Importantly, how each country envisages its patients engaging with the Networks depends upon their patient pathways and the nodes they have – thus each country has to develop its individual process in order to link ERNs to the national level and existing expertise. The advent of ERNs does *not* mean that patients should travel abroad for care more than they need to, but hopefully when this *is* necessary, the process will be facilitated. Certainly though, the main added value of the Networks will lie in enabling advice and expertise to travel more readily and more securely, enabling the generation and dissemination of knowledge through endorsed HCP and 'affiliated' partners to the national healthcare system. It is important to remember that **cross-border rights and responsibilities are already defined** as per the Cross-Border Healthcare Directive.

Maurizio Scarpa explained that amongst the many strengths of the ERN concept are the facts that many of the HCPs are already well-networked, and that the networks themselves have good geographical coverage and are patient-centred. However, when speaking of integrating ERNs with the national health systems there are major challenges ahead. Firstly, there is no standardisation of care across Europe – within the same country even, professionals work in different ways, and thus ERNs should attempt to create evidence-based guidelines to make treatments uniform within each country. Integration to the health systems would entail a sophisticated IT platform capable of interoperable data exchange with the national health services. The lack of sustainable funding also implies that ERNs are somehow 'outside' of the health systems.

Discussion: The participants clarified the fact that, although cross-border in scope, the ERNs are not seeking to create an international healthcare system – EURORDIS colleagues explained that this was not the spirit of ERNs, as patients and professionals are embedded in *national* health systems. The question perhaps is rather how MS will recognise those functions that will shortly exist through the ERNs, and realistically this entails financial support. EURORDIS colleagues further pointed out that **the Competent National Authorities of the MS and EEA countries have a major role to play here, beyond the endorsement of centres – they will need to determine how to connect ERNs with the other areas of national policy and with national structures.**

When discussing financial support for ERNs, as a means of embedding appropriately in national health systems, many of the ANCs were keen to emphasise that one must envisage not only the coordination costs -which will be substantial- but also the costs of virtual consultations and virtual care provision: there is already a system in place to reimburse the costs of a patient travelling for care, for instance to receive a specific procedure. However, some insist that this expert advice function should also be reimbursable, that this service cannot be provided for free. Participants discussed the prospects of adding an option under the S2 function, for instance. Furthermore, the legal status of ERNs was identified as a significant concern by several of the ANCs: it is understood that ERNs themselves will not be legal entities (beside the legal consideration of ERNs established in the legal base[6]). The HCPs *are* of course legal entities, but some of the ANCs are concerned about legal responsibilities of coordinators and HCPs when discussing medical matters and patient cases at the ERN level.

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[6] Commission Implementing Decision (2014/287/EU) and Delegated Decision (2014/286/EU)

The absence of a legal identity for the ERN itself raises challenges for the networks in terms of securing funding. Some ANCs fear this will limit the impact of the Networks, that the ERNs will have to limit their activities and thus could not be evaluated against the criteria envisaged at present. The EC colleagues explained that the goal is to secure coordination funding for all Networks approved as ERNs. They also reminded the group that the scope and goals of the Networks are already established, as are the rules under which -at present at least- all stakeholders must operate. To seek to change the social security Regulation would take many years, as the social security committee will wish to see the concrete tasks, the benefits, the costs etc. Therefore, the EC suggested, the best way to proceed would be to adopt a strategic, stepwise approach, building confidence across MS and providing proof of concept of ERNs in the formative years of their establishment.

Helena Kääriäinen briefly introduced the focus of the WG she is chairing in the BoMS, concerning Conflicts of Interest/Industry interactions. The ANCs stressed that the Networks cannot exclude industry collaborations, as Industry are a partner and stakeholder in rare disease healthcare and research. It was agreed that this is not the intention of the paper being generated by the Board; Industry should be excluded from the decision-making process around guidelines etc., for obvious reasons, but RD-ACTION partners emphasised that there is more to this topic than the risks, and the conflict of interest issue. It will be important for ERNs to contribute to the generation of real world evidence for new therapies and innovation, by providing quality data. It was agreed by the group that the paper under generation by the BoMS WG should demonstrate a balanced approach. Helena has approached RD-ACTION for support in disseminating a number of key questions to the ANCs, and possibly discussing these via a teleconference in early November, to enrich the work the Board is doing in this area. RD-ACTION confirmed it would be happy to support this collaboration.

Conclusions and Action Points:

It will be necessary to closely link ERNs to national healthcare systems, through the active collaboration of MS Competent National Authorities and HCP clinical leads – this will be easier where countries have mapped their expertise in the provision of highly specialised healthcare and have developed clear pathways through these systems, but in all cases we should think of building ERNs from the strong foundation of national health systems.

Regarding the membership and affiliation of centres with ERNs, the difficulties on both sides of this debate were acknowledged – although this is a national prerogative, as defined in the legal base, several ANCs and RD-ACTION colleagues requested some degree of collaboration here, and an opportunity for the ANCs to at least share their views on this somehow (e.g. through a teleconference with the WG). It was agreed on both sides that to avoid disharmony in the field, it is crucial to define and announce the procedure and timeline for the assessment of new HCPs with national endorsement wishing to join existing networks, *and* timelines and a strategy for the endorsement of 'affiliated' partners.

RD-ACTION will distribute a list of questions from the WG on Industry Interactions, as requested by Helena, and will follow this up with a teleconference in November.

Given the range of topics to discuss in the coming months, it was proposed by a BoMS representative that the interaction between the BoMS and the ANCs should be formalised: DG SANTE explained that once the Networks are approved, the EC will be able to organise formal meetings directly, for instance back-to-back with Board meetings. In the meantime, the Joint Action confirmed it could continue to support this process through teleconferences and perhaps another face-to-face meeting[7]- although probably without budget for participants.

To close the meeting, it was confirmed that the report will be share with all the ANCs and the full Board of MS (the latter via DG SANTE communication channels). To enable participation of the patient representatives and project topic experts, it was not possible to invite all of the Board Members to join the workshop following the meeting; however, the Chairs from this morning's discussions (Till and Aikaterini) plus the Lithuanian representative Birute (as host of the next ERN Conference) will participate, and should be able to provide feedback to the wider Board, if necessary. The presentations and outputs of the workshop will also be made publically available in the coming weeks. Victoria thanked everyone for their contributions and closed the meeting.

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[7] One priority would likely be, as above, the key topic of ERN membership and affiliation: during the meeting, it was pointed out that 67% of the HCPs currently under consideration for membership of the ERNs come from just five countries

Agenda and Participant List

9:00 Welcome (Victoria Hedley, RD-ACTION)

9:10-9:20: *ERN Proposals: the Status Quo* Enrique Terol and Anna Carta

9:20-10:50: Discussion Sessions:

1. Membership coverage and 'affiliation' to ERNs (Discussion Chairs K. Damigou and L. Sangiorgi)
2. Integration of ERNs with national health systems (Discussion Chairs T. Voigtländer and M. Scarpa)

10:50: Next steps for collaboration between the Network Coordinators and the Board of MS

11:00 Meeting Ends

Board of Member States of ERN participants

REPRESENTATIVE NAME	COUNTRY
Till Voigtländer	Austria
Violetta Anastasiadou	Cyprus
Helena Kääriäinen	Finland
Patrice Dosquet	France
Aikaterina Damigou	Greece
György Pfliegler	Hungary
Ms Judit Pako	Hungary
Birute Tumiene	Lithuania
Katarzyna Kotulska- Jóźwiak	Poland
José Alexandre Diniz	Portugal
Ines Palanca Sanchez	Spain
Laura Marin	Spain
Colin Pavelin	UK

European Commission and ERN Tender Participants

PARTICIPANT NAME	AFFILIATION
Anna Carta	DG SANTE B3 (Cross-border Health Care and eHealth)
Caroline Hager	DG SANTE B3 (Cross-border Health Care and eHealth)
Markus Kalliola	DG SANTE A4 (Information Systems)
Hélène Le Borgne	DG SANTE B3 (Cross-border Health Care and eHealth)
Simona Martin	Joint Research Centre
Enrique Terol	DG SANTE B3 (Cross-border Health Care and

	eHealth)
Ana Rodriguez	Agencia de Calidad Sanitaria de Andalucía – contractor for *the* ***Independent Assessment Body to perform the assessment of the eligible ERN applications***
Jaroslaw Waligora	DG SANTE C1 (Health programme and chronic diseases)

Joint Action Participants

PARTICIPANT NAME	**AFFILIATION**
Valentina Bottarelli	RD-ACTION/ EURORDIS
Victoria Hedley	RD-ACTION
Matt Johnson	RD-ACTION/ EURORDIS
Yann Le Cam	RD-ACTION/EURORDIS

Applicant Network Coordinators (or their Representatives)

PARTICIPANT NAME	**PROPOSED THEMATIC GROUPING REPRESENTED**	**NAME OF PROPOSED ERN**
Michelle Battye	Rare Urogenital Diseases	eUROGEN
Melanie Brunhofer	Paediatric Cancer ERN	PaedCanERN
Helen Cross	Rare & Complex Epilepsies	EPI-CARE
Sofia Douzgou	Rare Malformations and Developmental Anomalies and Rare Intellectual Disabilities.	ITHACA
Pierre Fenaux	Rare Haematological Diseases	eurobloodnET
Holm Graessner	Rare Neurological Diseases	ERN-RND
Marine Hurard	Rare Multisystemic Vascular Diseases	VASCern
Ruth Ladenstein	Paediatric Cancer ERN	PaedCanERN
Dorothée Leroux	Rare Eye Diseases	ERN-EYE
Ansgar Lohse	Rare Hepatic Diseases	ERN-LIVER
Eduardo Lopez Granados	Transplantation (SOT & HSCT) in Children	TRANSCHILD
Maria Madrigal Montero	Rare Immunological and Autoinflammatory Diseases	RITA
Maria Manu	Rare Haematological Diseases	eurobloodnET
Alberto Pereira	Rare Endocrine Diseases	ENDO-ERN
Luca Sangiorgi	Rare Bone Diseases	BOND
Georgia Sarquella-Brugada	Rare Cardiac Diseases	GUARDHeart
Maurizio Scarpa	Rare Hereditary Metabolic Diseases	MetabERN
Franz Schaefer	Rare Renal Diseases	ERKNET
Christoph Schramm	Rare Hepatic Diseases	ERN-LIVER

Rachel Thompson	Rare Neuromuscular Diseases	EURO-NMD
Thomas Wagner	Rare Pulmonary Diseases	ERN-LUNG
Rene Wijnen	Rare Gastrointestinal Diseases	ERNICA