Deliverable 6.3 (Part 2): Workshop Report from EU Level Workshop

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Introduction

Rare 2030 is a foresight study requested by the European Parliament and supported by the European Commission to gather input and propose policy recommendations that will lead us to improved policy and a better future for people living with a rare disease in Europe in the next 10 years and beyond. This two-year project will end with a presentation to the European Parliament during Rare Disease Week, February 2021, with recommendations on the most critical areas requiring robust policies. Fig. 1 below illustrates the four main stages of this foresight study.

In Summer/Autumn of 2020, the project was moving from the **scenario phase (3)** to the elaboration of **policy recommendations** (4) to guide the rare disease field towards the future deemed most preferable (that is to say, Scenario 1, 'Investment for Social Justice' https://www.rare2030.eu/scenarios/)

In drafting these future-facing policies it was always the plan to cast a particular focus on the future for European Reference Networks (ERNs), given their central importance to the European rare disease and specialised care community. In lieu of organising a physical event in 2020 to support this activity, **four 2.5-hour workshop sessions were staggered across a period of several weeks**. The sessions were designed to collectively constitute one whole workshop, and each session focused on an area of major strategic interest to ERNs. These were followed by a closing plenary on 26th October to present the main conclusions from all sessions.

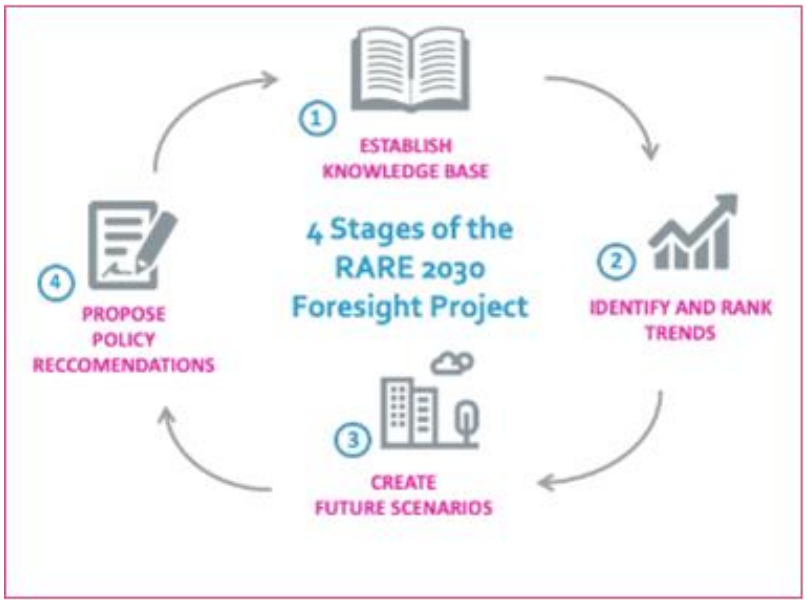


Fig. 1: The 4 Stages of Rare 2030

The Rare 2030 Foresight Study and the resulting recommendations cast a particular focus on the future for European Reference Networks (ERNs), given their central importance to the rare disease community. Rare 2030 partners organised 4 parallel workshops, divided into four 2.5- hour breakout sessions staggered across several weeks in September and October 2020. These were followed by a closing plenary on 26th October to present the main conclusions from all sessions.

Each of the four working sessions focused on an area of major strategic interest to ERNs, as below.

Workshop 1: 21 September 2020

Governance and strategic positioning of ERNs.

Should the ERNs have a legal status, and if so, what is the best route/best way to achieve this? How can we secure financial sustainability of ERNs? Future composition of ERNs (in terms of model and scale): can we

reach the right balance between Centres directly and indirectly involved in the ERNs? How should ERNs collaborate with stakeholders and countries *externally* (outside of the EEA – if indeed they should)

Workshop 2: 28 September 2020

Integrating ERNs to national systems and frameworks

What is the best way to integrate ERNs into national health systems? How should ERNs complement the wider national landscape of Centres of Expertise for RD? What role do you see ERNs playing in bridging health and social care? How should HCPs and 'affiliated' partners sit within the national ecosystems? Should ERNs drive future policies at national level, and if so, how?

Workshop 3: 29 September 2020

Role of ERNs in virtual care delivery and cross-border healthcare

How can the CPMS transform virtual care for specialised conditions, and how can the ERNs more widely accelerate positive telemedicine trends towards balanced physical-virtual clinics of the future? What does success look like for you? Is it improving the health-related outcomes for patients visiting member HCPs or for whole populations? How can we receive legal/regulatory/financial recognition of time and expertise spent on cross-site CPMS case discussions? Could/should decisions of ERN panels bear more weight?

Workshop 4: 12 October 2020

ERNs, research, and the data ecosystem of the future

What should be the ERNs' 'data strategy' in 2030? How can ERNs contribute to diagnostic equality across Europe? What is the best, most realistic role for them to play? How do we want ERNs to be positioned, research-wise? How do we review the policies (and address the problems) around data-sharing and health & research?

Participation to the Workshop Sessions

The 4 workshop sessions together involved 73 individual participants, including ERN representatives, ERN Hospital Managers, ePAG advocates, EURORDIS experts, Board of Members States of ERNs representatives, key members of the European Commission, and partners of the Rare2030 project.

The breakdown of stakeholders was as follows: (Fig 2: shows workshop composition visually)

ERN Coordinator/ERN Healthcare Provider Representative	28
ePAG Advocate	18
Rare2030 Representative	14
Board of Members States of ERNs	3

ERN Hospital Manager	5
European Commission	5

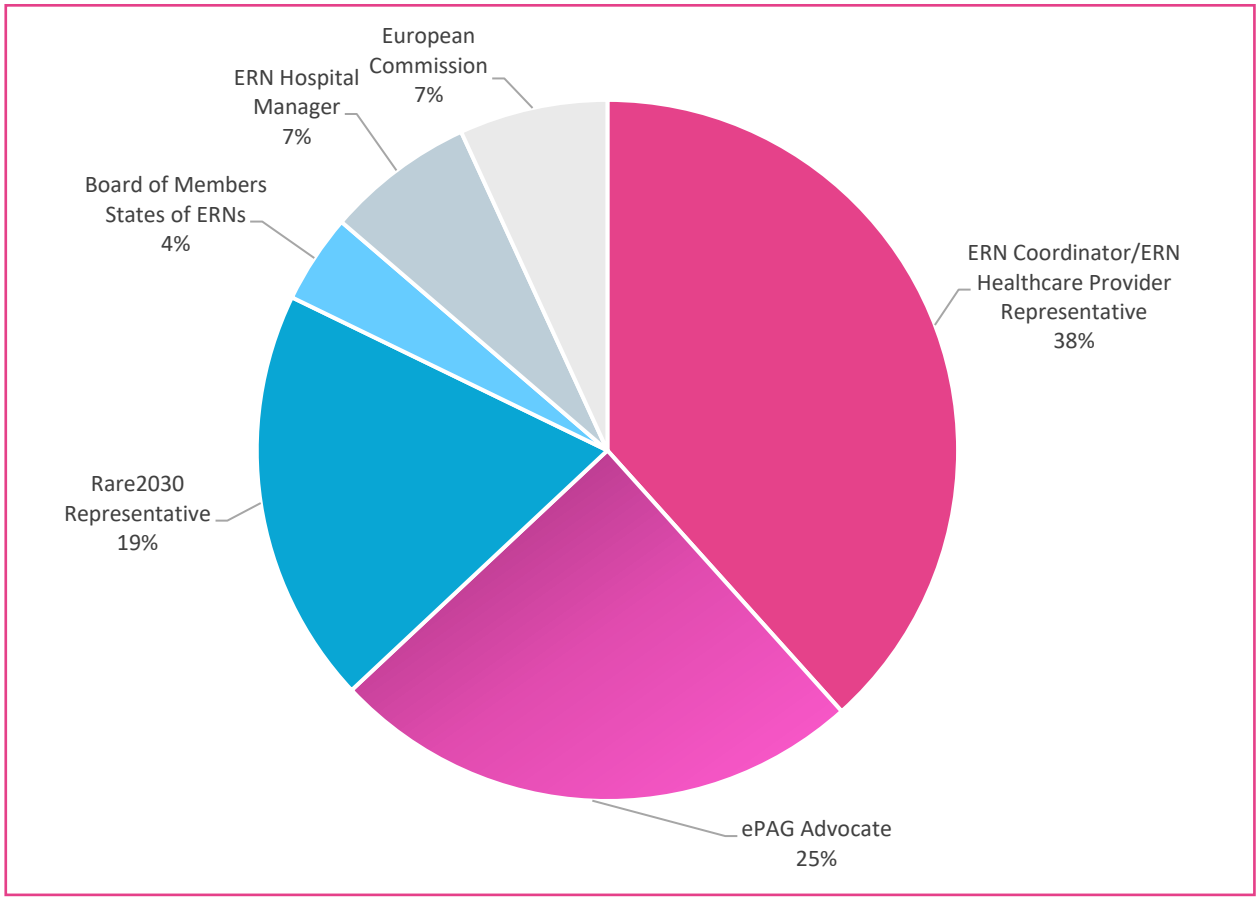


Fig. 2 Composition of the ERN Workshop Sessions

Structure of the Workshop Sessions

Each session centred on a number of proposed preliminary recommendations directed towards ERNs. In total, across the 4 sessions of the back-casting workshop, over 80 preliminary recommendations were discussed.

Before each workshop, participants received an agenda (Fig 3 below) and overview of the major strategic topics to be discussed. Support to workshop organisation and facilitation was provided by *Wild Is The Game*. A novel aspect the facilitators brought to the workshop was the use of a virtual, interactive platform – MIRO. Rare2030 had previous experience of using this platform as part of the Rare2030 Young Citizens Conference held in July 2020. To encourage engagement with MIRO, opportunities were provided for

practice with the platform before workshops and as part of the introduction to each session an interactive exercise was arranged.

In addition, a series of online meetings were held with the facilitators and scribes (predominantly Rare 2030 partners) to prepare them for their role and ensure smooth execution of the workshop sessions. These meetings were supplemented with a dedicated video for facilitators and scribes to review, which summarised their workshop roles and demonstrated how to work with the bespoke MIRO templates.

Shortly before each workshop, participants were divided into smaller breakout groups, typically involving six-eight people. Participants were allocated to a group by the UNEW coordinators, to ensure a mix of roles and experience in each. Each group had a facilitator and scribe.

Time (CEST)	Activity	What will happen?
15.30	*Zoom call will be open for those wishing and able to join early*	*Wild is the Game (our professional facilitation company) will be available to welcome you as you join. It's helpful if you rename yourself on Zoom with name and stakeholder category, e.g. Dorica Dan, ePAG, so we see who everyone is*
16:00	**Session begins: Welcome and short presentation**	Victoria Hedley will explain the aims of this session and how this workshop fits together. She will explain what outputs we wish to have and how your inputs will be used.
16:10	Quick digital 'immersion'	Olivier Percevaut (*Wild is the Game*) will demonstrate our bespoke Miro platform working space
16:20	Individual exercise (adapted since session 1!)	The ERN community proposed 3 key strategic questions ('topics') to drive this session: A. How can the CPMS transform virtual care for specialised conditions, and how can the ERNs more widely accelerate positive telemedicine trends towards balanced physical-virtual clinics of the future? B. What does success for the ERNs look like for you, in terms of 'care'? Is it improving the health-related outcomes for patients visiting member HCPs or for whole populations? C. How can we receive legal/regulatory/financial recognition of time and expertise spent on cross-site CPMS case discussions? Could/should decisions of ERN panels bear more weight (e.g. in terms of influencing decision-making on patients accessing treatments domestically or abroad?) In a change to session 1, by the end of Friday 25th you will receive a list of preliminary draft recommendations, for you to review in advance. You will see these *same* recommendations on the Miro board at this point, and will be asked to use this time to familiarise yourself again and we conduct a simple vote to give an **approximate** idea of those the group

		sees as most important, strategically, for ERNs to function well and add value in the future.
16:35	Group Working: developing or discussing potential ERN-focused recommendations	The participants will split into groups of up to 10 people and will work together in greater depth to address ONE of the three topics. We will further develop the draft recommendations explored above, identifying areas of consensus and disagreement, capturing comments and exploring how some of these recommendations could be implemented (thinking ambitiously and long-term!) If a group wishes, and time allows, they can move onto a second question. If you wish to facilitate a group, let Vicki know!
Ca. 17:25	Short Break	
Ca. 17:30	Reporting back	The full group will come back together and rapporteurs for each group will report back on some of their main conclusions and any recommendations they were able to already propose/support.
18:20	Quick satisfaction poll and next steps	We will use any feedback to help shape the remaining working session We will end with a closing message and a reminder of how this session links to the others
18.30	Session ends	

Fig 3: Example Workshop Agenda

The model for each workshop was the same:

Introduction

The sessions began with a short presentation, reminding participants of the aims and the context in which this work was taking place. The first session began with a slightly longer presentation, delivered by Yann le Cam and Victoria Hedley, and sought to position this staggered event within the broader rare disease policy landscape. Each gave an overview to the Rare2030 project and clarification as to the current phase (back-casting exercise to identify potential recommendations). There was a short introduction to the strategic topic to be focussed upon e.g. Integrating ERNs to national systems and frameworks. A brief MIRO practice exercise was then delivered, involving participants creating post-it notes and adding comments in response to a relevant test question.

Ranking Preliminary Recommendations

The introductory 'digital immersion' exercise was followed by a time limited task. The participants had up to 20 minutes of solo working time, to review a number of draft recommendations (approx. 20 each time) which had been proposed for them and which were laid out on the MIRO board. For workshop sessions 2, 3

and 4, this list of draft recommendations was actually circulated *in advance* of the sessions, to allow people to familiarise themselves with the content (this was a change from session 1, and was implemented following a debrief on the part of the organisers with *Wild is the Game*, to make the changes needed to optimise the delivery of the other 3 working sessions). The draft recommendations themselves had been elaborated by UNEW with support from Rare 2030 partners, particularly EURORDIS. The participants were asked to vote for a limited number (usually 12) of those approx. 20 recommendations, and to comment upon as many as they wished. These individual scores were then automatically combined to indicate a ranking. An example of the output from this exercise can be seen in Figure 4 below. The green post it notes on the left are highlighted with a ranking score.

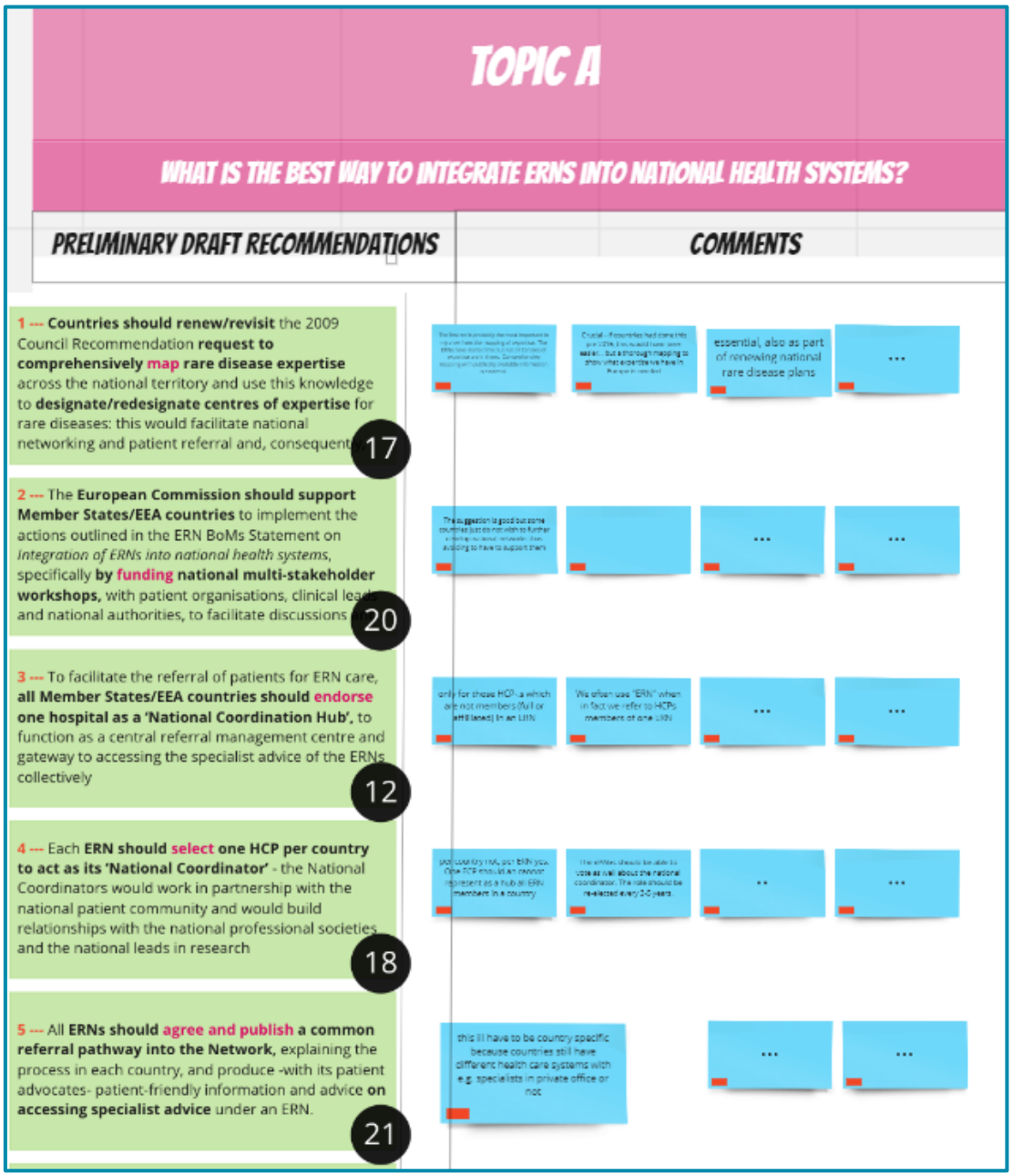


Fig. 4 Example of recommendation 'scoring'

Group Exercise

Each workshop centred on a series of three or four key topic-related questions which would form the basis for these group discussions. One key question was allocated per break out group. For example, in workshop 4 -focussed on **ERNs, research, and the data ecosystem of the future -** the group 'topic' questions were:

- What should be the ERNs 'data strategy' in 2030? How do we review the policies (and address the problems) around data-sharing and health & research?
- How can ERNs contribute to diagnostic equality across Europe? What is the best, most realistic role for them to play?
- How do we want ERNs to be positioned, research wise? (is the research side supported strongly enough, by all countries and actors? How should ERNs engage with industry in the future?

Participants, in break-out groups, groups brainstormed on the feasibility of each recommendation, with the reminder to think ambitiously. Wherever possible, the participants proposed steps which would be needed to implement some of these strategic recommendations. MIRO was used to capture ideas, identifying areas of consensus and disagreement, capturing comments in a visual way using 'post-it' notes, colours and drawn lines to indicate relationships. The groups worked on templates (see Fig. 5 and 6 below)

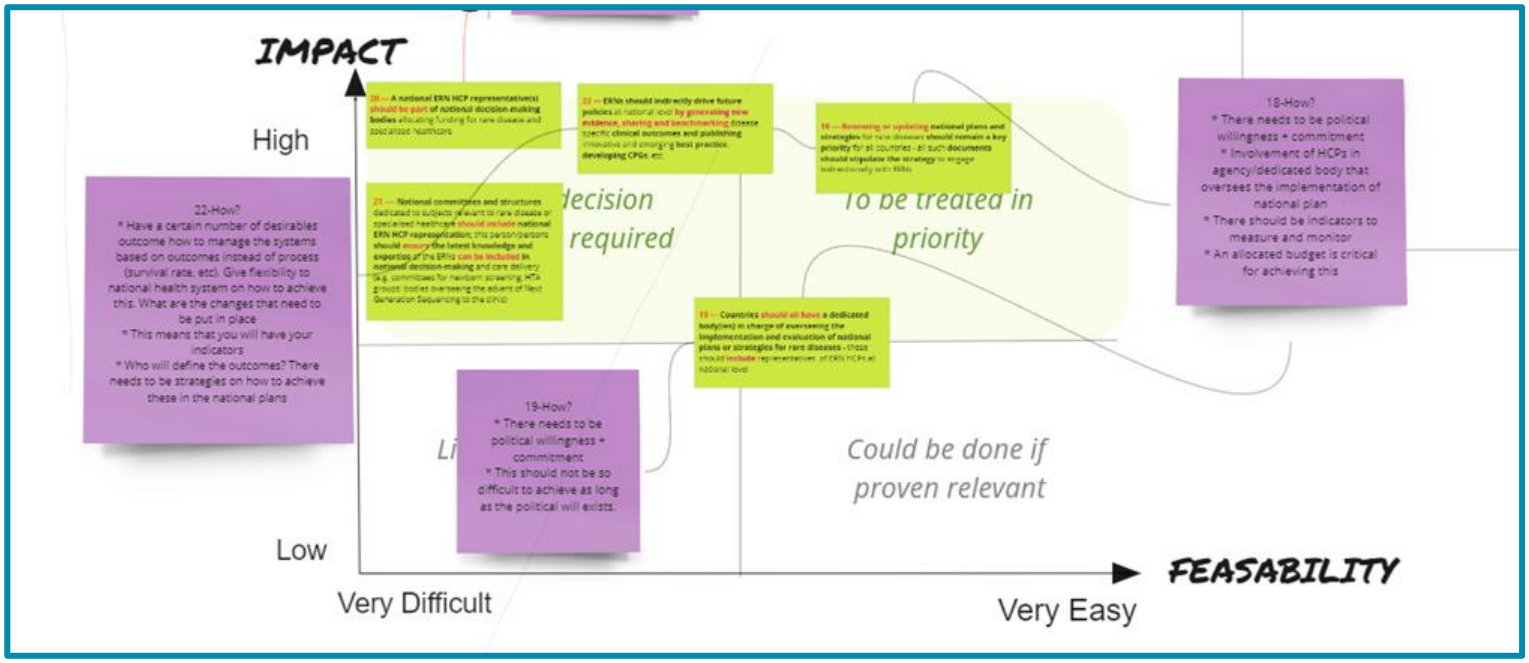


Fig. 5 Example Group Work Template

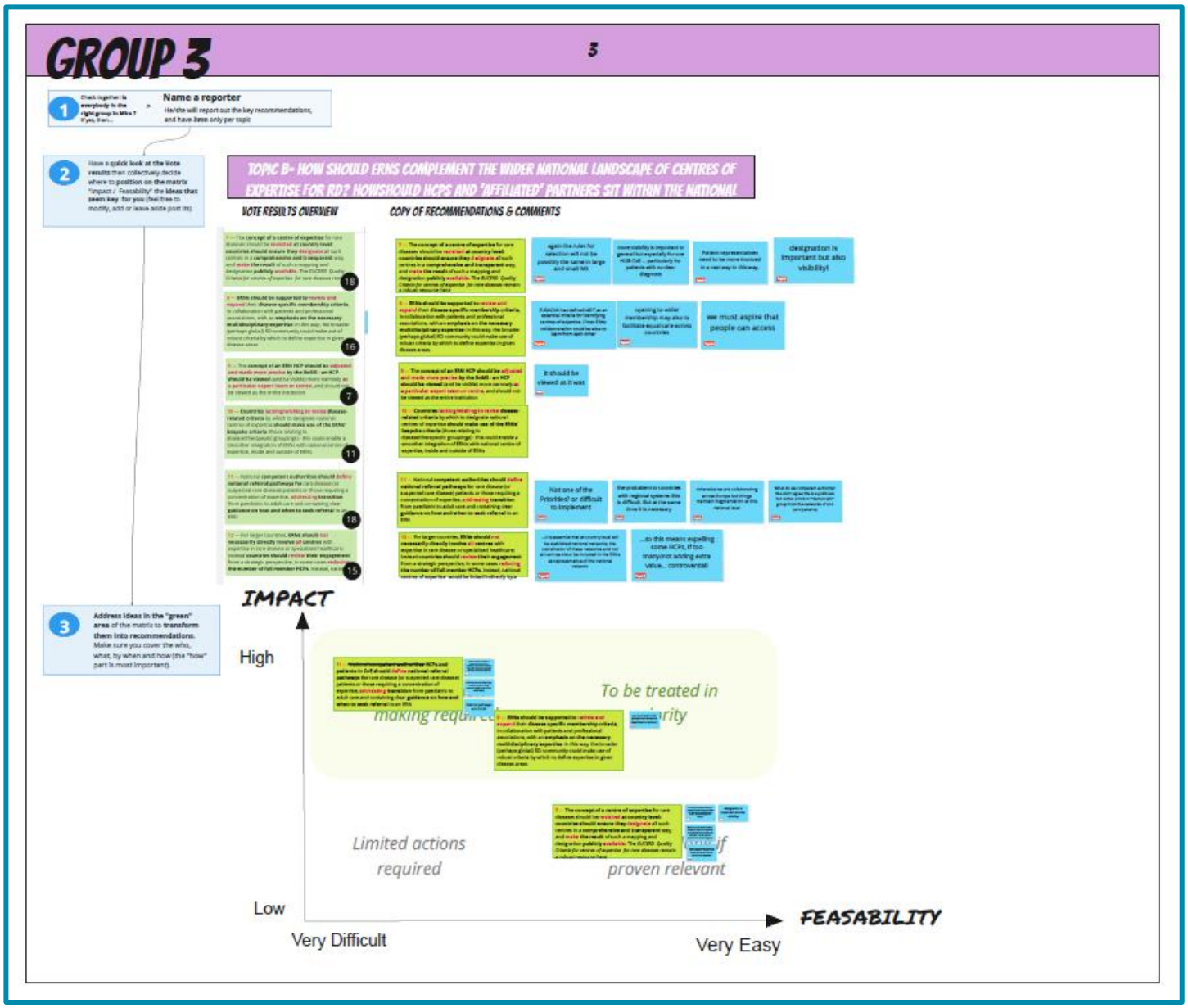


Fig. 6 Example Group Work Template

Reporting Back

The full group came back together and rapporteurs for each group reported on some of their main conclusions and highlighted recommendations they were able to strongly propose/support.

Next Steps

Participants completed a short evaluation survey with instantaneous results, followed by a brief 'next steps' outline

Plenary Event

These working sessions were followed by a **Closing Plenary on 26 October 2020,** to present the main conclusions from all sessions and open up participation to a wider audience.

The recording of this event can be found here: https://www.rare2030.eu/key-events/26-oct-2020-ern-plenary-event/

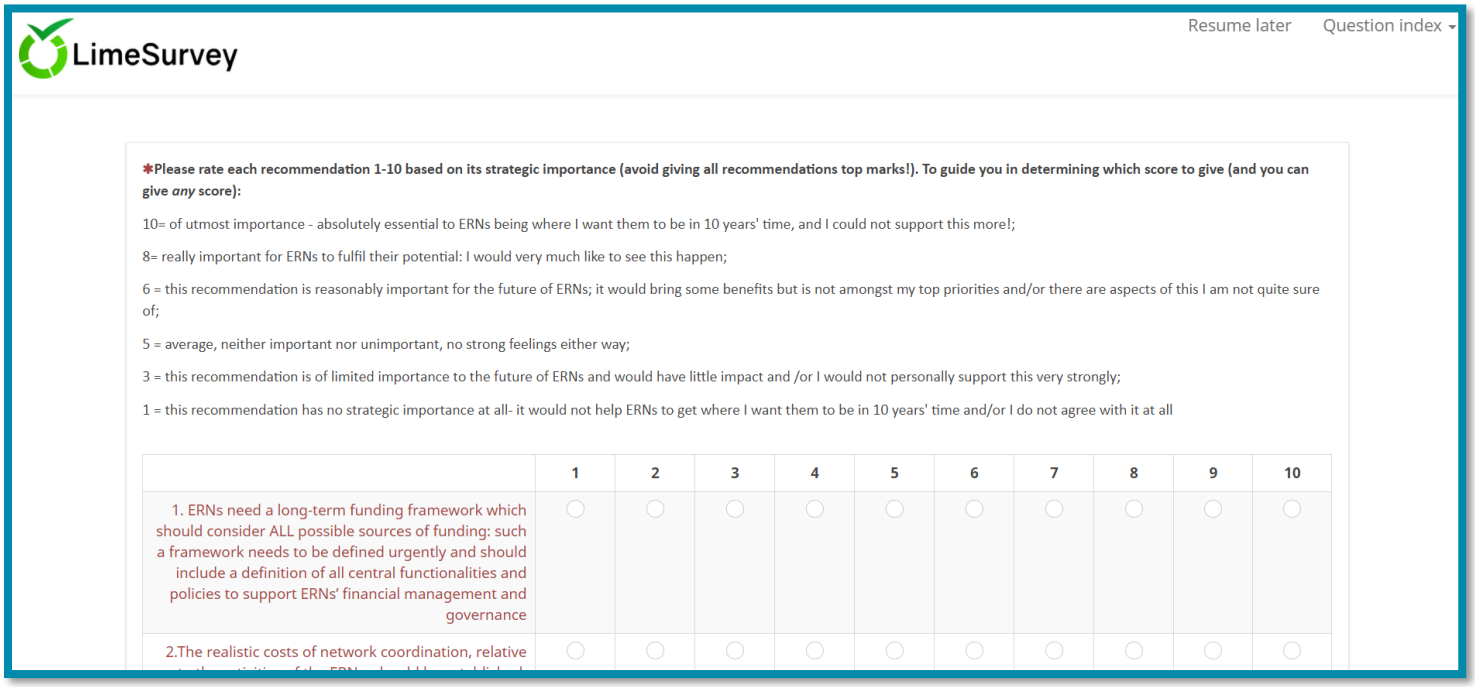
After the final working session, the recommendations deemed most relevant had been extracted - and amended where necessary - and were incorporated to a survey (featuring 33 recommendations in total – see below). This survey asked respondents to consider all 33 and rank them from 1-10 in terms of strategic importance to the future of ERNs. In this way, the Rare 2030 partners would be able to identify more objectively which recommendations should be prioritised in the policy- making activities of the coming months and years. The survey was disseminated to all workshop participants, and the preliminary results were shared during this Plenary event, to enable experts to provide their reactions and responses to these recommendations and how to implement them.

Following the Plenary, the recommendations survey remained open to participations, but was also shared more widely with the broader rare disease community, in particular through ePAG communication channels, through individual ERN communications to their respective centres, and to the partners of the European Joint Programme for Rare Disease Research.

Results of the Recommendations Survey

The survey centred entirely upon the 33 recommendations (see Annex 1): participants were asked to rank each in terms of the strategic importance. Respondents were strongly advised to avoid simply granting the highest numbers to *all* recommendations, and were instead urged to use discretion in their scoring, to really highlight the recommendations they personally felt were of greatest important for enabling ERNs to fulfil their potential and exist in an ecosystem compatible with the preferred Rare 2030 scenario (I.e. Scenario 1, 'Investment for Social Justice').

The survey interface appeared as follows:



The survey took approximately 20 minutes to complete, and in the end, 200 full responses were received (discarding 4 duplicates). The survey presented recommendations in their workshop categories, in an effort to cluster 'like with like' and ease completion for the respondents. The first set were those proposed and elaborated under the working session 'Governance and Strategic Positioning of ERNs', then the next set stemmed from the second session of 'Integrating ERNs to National Systems and Frameworks' etc.

The table below presents the final ranking and shows the total 'score' each recommendation received. They are colour coded, to illustrate, loosely, the focus of the recommendation (e.g. is it a data/research related recommendation, or one more concerned with the strategic positioning of ERNs?)

‘Governance and Strategic positioning of ERNs’
Integrating ERNs to national systems and frameworks’
‘Role of ERNs in virtual care delivery and cross-border healthcare’
‘ERNs, research, and the data ecosystem of the future’

Recommendation	Score in Survey
ERNs need a long-term funding framework which should consider ALL possible sources of funding: such a framework needs to be defined urgently and should include a definition of all central functionalities and policies to support ERNs’ financial management and governance	**1791**
The concept of a Centre of Expertise for rare diseases should be revisited at country level: countries should ensure that the concept of a centre of expertise within the national territory (including ERN HCPs) is as aligned as possible with the *EUCERD Recommendations on Quality Criteria for Centres of Expertise for Rare Diseases*, including also the requirements to ensure multidisciplinarity and to collaborate with paramedical, social, and educational actors. Countries should ensure they designate all such centres in a comprehensive and transparent way, and make the result of such a mapping and designation publicly available, demonstrating how ERN HCPs and ‘affiliated’ centres fit within wider national networks (where applicable).	**1604**
Disease-specific registries (where positively evaluated by ERNs based on rigorous criteria OR created anew by ERNs in future) should be interoperable with the new ERN registries and any robust national RD registries: these should all be connected (sustainably) to ERDRI and the European Joint Programme for Rare Diseases Virtual Platform to provide a fully functioning registration ecosystem	**1592**
Renewing or updating national plans and strategies for rare diseases should remain a key priority for all countries - all such documents should stipulate the strategy to engage bidirectionally with ERNs: support in this task should be provided by a group/body with a remit to encompass all RD topics, beyond ERNs alone	**1592**
European Reference Networks should be specifically and adequately funded to develop and conduct natural history (and where possible accompanying biomarker) studies, a	**1592**

minimum of 5 every 2 years, to build the knowledge base and capacity for clinical research in disregarded diseases/areas lacking research	
Each Member State/EEA should define a mechanism, centred upon Orphanet, for instance, to disseminate and utilise the knowledge and evidence generated by the ERNs, to impact across the wider health and social systems: in particular, clinical practice guidelines/clinical decision support tools generated or endorsed by an ERN should be fully applied in all Member States and EEA countries, and national committees and structures dedicated to rare disease or specialised healthcare should include some level of national ERN HCP representation	**1584**
The European Commission should support Member States and EEA countries to implement the actions outlined in the *ERN BoMS Statement on Integration of ERNs into national health systems*, specifically by funding national multistakeholder workshops, with patient organisations, clinical leads and national authorities, to facilitate discussions and actions on integration into each of the national health systems.	**1584**
European Reference Networks should receive earmarked -and adequate- funding through European programmes to conduct clinical research and trials (involving centres inside or outside of the Networks, as required) and to research neglected topics including rehabilitative, holistic and social research	**1583**
The European Commission should provide coordination funding to coordinating HCPs but Member States /EEA countries should provide funding to each national HCP/affiliated member within their national territory (providing they meet performance and impact indicators) to support their engagement in ERN activities	**1575**
National competent authorities should define national referral pathways for rare disease (or suspected rare disease) patients or those requiring a concentration of expertise, addressing transition from paediatric to adult care and containing clear guidance on how and when to seek referral to an ERN; ERNs should then compile and publish these pathways, explaining the process in each country and producing -with its patient advocates- patient-friendly information and advice on accessing specialist advice under an ERN	**1558**
Countries should be encouraged to revisit and update their national designation of Centres of Expertise (CEs) for rare diseases and strengthen the organisation of national rare disease	**1551**

and specialised care networks - this should then translate to a more strategic engagement of national CEs with ERNs, via a limited number of full member HCPs.	
Data from hospital EHR (electronic health record) systems should be interoperable with the ERNs' Clinical Patient Management System (CPMS), and with ERNs' new epidemiological registries, allowing minimal data entry and maximum automation (with accompanying quality assurance) - all such systems should be aligned with the European Health Data Space.	**1548**
The realistic costs of network coordination, relative to the activities of the ERNs, should be established, and coordination funding provided on these grounds – a core coordination budget, available to all ERNs, should be supplemented by an additional variable budget, based on size, scale, coverage and activities	**1546**
ERNs should develop clear and transparent rules for patient engagement, adequately supporting the involvement of patient organisations and their representatives in the different ERN activities, and should fairly compensate patient representatives for expenses and expertise.	**1537**
The Clinical Patient Management System (CPMS) should be fully compatible with any referring HCP systems, enabling automatic and two-way cross-talk with Electronic Health Records, to populate and update records post case referral: CPMS data should be fully searchable, and cases accompanied by an appropriate PPRL (privacy preserving record linkage) solution	**1532**
The Coordination and Support Action funded by the H2020 programme to support the creation of Clinical Research Networks (covering 4 domains: clinical research (including PCOMs); data management; engagement and dissemination; and administrative support) should be supplemented by additional funding to deploy core services to become fully operational by 2025	**1531**
ERNs should gather and create, in collaboration with patient organisations, resources and data which could support rare disease patients in receiving more and better adapted integrated and more personalised care in their local environment: such resources should	**1526**

translate to heterogenous care and social settings, by focusing on clarifying and explaining the (often poorly-understood) needs of patients with complex conditions, and adaptations/approaches which could help patients (making use of digital tools where needed and helpful)	
ERNs should be financially supported to co-create (together with the European Joint Programme for Rare Diseases) a comprehensive data strategy and implementation plan by 2023, envisaging the necessary activities across 6 action lines: architecture - cloud computing services and IT support for registries and other databases; data collection protocols; data curation services; data management tools (services and tools to search, access and share data, tools to manage own data); data analytics tools and services; and a data governance framework.	**1500**
ERNs should develop strategies to minimise disparity between European regions in access to high quality healthcare: disease-related metrics should be agreed and monitored	**1499**
The Social Security Regulation and/or Cross-Border Healthcare Directive should be amended to allow for payment of time spent on cross-border virtual consultations performed through the CPMS, following a systematic national referral process	**1488**
The future ERN health data strategy must be anchored to the wider European health data and IT ecosystem, driven by a concerted policy action of all the relevant DGs and aligned with national health data strategies from the majority of MS/EEA countries; in this context, ERNs should help to shape the future Health Code of Conduct for secondary use of data (addressing the need to make GDPR research-friendly)	**1486**
An efficient project/tender should be funded to establish a pricing model to reimburse expert time spent on CPMS case review and propose options for payment (e.g. a *quid pro quo* system, a straightforward billing of another Member State/EEA country (perhaps with a differential GDP-based pricing scheme), a reduction in the workload of ERN HCP clinicians in lieu of payment for CPMS reviews, etc)	**1480**
European Reference Networks and the Board of Member States should embrace a strategic mission of promoting more integrated care, encompassing integration of different medical specialities, but also of paramedical and social actors, in line with the *EUCERD*	**1479**

Recommendations on Rare Disease European Reference Networks and the *Commission Expert Group for Rare Diseases Recommendations to Support the Incorporation of Rare Diseases into Social Services and Policies*	
ERNs should sit at the centre of all future efforts to refine and evolve all ontologies and standards for data collection and utilisation into a common data model, including efforts to facilitate extraction and mining from real-world data	**1464**
ERNs should be supported to review and expand their disease-specific membership criteria, in collaboration with patients and professional associations, with an emphasis on the necessary multidisciplinary expertise: in this way, EU countries (perhaps even the global RD community) could make use of robust criteria by which to define expertise in given disease areas	**1463**
The future ERN data strategy must be targeted towards all rare disease patients in Europe, and not only those attending ERN HCPs: opportunities must be created for patients to foster robust data partnerships, determine governance, and contribute/extract data to or from appropriate registries, care records and other relevant data sources.	**1460**
Countries should consider automatically authorise requests for treatments or therapies if deemed beneficial by an ERN panel through CPMS: the ERNs' expertise should hold more weight than national expert bodies who make such decisions at present	**1451**
A dedicated study/project should be funded, to support countries in developing their Electronic Health Records and virtual care delivery services to best address the specificities of rare diseases and highly specialised healthcare, and promote interoperability: this could aim at wider national deployment of the CPMS or a system compatible with it, as the basis for virtual care provision for complex rare disease cases nationally (whilst ensuring that any move towards more virtual care must be proportionate, to avoid further marginalization of a vulnerable population).	**1445**
A cross-ERN working group on integrated and holistic care should be established (by 2025), in partnership with RareResourceNet (the European Network of Resource Centres for Rare Diseases), as a gateway to build joint guidance on collaborative approaches for the	**1393**

provision of integrated and holistic care to people living with a rare disease: dedicated funding should be made available for broad stakeholder meetings and activities to advance this goal	
A common EU agency should be created/adapted to enable ERNs to operate more flexibly and effectively, and receive funding from a range of sources (including 'external' sources such as industry and private donors, with an appropriate governance for public-private partnerships)	**1390**
All Member States and EEA countries should identify and publicise a clear process to facilitate the referral of patients for ERN care: this might include endorsing one centre as a 'National Coordination Hub' (or, if a federated system, endorsing a centre in each region, or in several strategically-selected regions) to manage referrals and function as gateways to accessing the specialist advice of the ERNs collectively - any such centre should work in partnership with the national patient community, and build relationships with national professional societies and research leads	**1353**
A special category of association or collaboration or affiliation should be created to allow formal collaboration and recognition of centres from countries outside of the EU Member States /EEA; clear rules on shared activities (what is and is not permitted) should be created	**1342**
ERNs should ideally each be legal entities they should – as an interim solution, at least - they should be nested within an organisation such as a foundation, to provide a mechanism for ERNs to easily receive funds.	**1293**

Annex 1: Full list of Recommendations included in the Survey

The recommendations included to this survey can be viewed below (grouped by strategic area, in line with the four working sessions)

SESSION 1 – GOVERNANCE AND STRATEGIC POSITIONING OF ERNS

1. ERNs need a long-term funding framework which should consider ALL possible sources of funding: such a framework needs to be defined urgently and should include a definition of all central functionalities and policies to support ERNs' financial management and governance
2. The realistic costs of network coordination, relative to the activities of the ERNs, should be established, and coordination funding provided on these grounds – the amount of funding available for coordination-related activities should differ between ERNs, based on scale and coverage
3. A common EU agency should be created/adapted to enable ERNs to operate more flexibly and effectively, and receive funding from a range of sources (including 'external' sources such as industry and private donors, with an appropriate governance for public-private partnerships)
4. ERNs should ideally each be legal entities – in the interim, they should be nested within an organisation such as a foundation, to provide a mechanism for ERNs to easily receive funds.
5. Countries should be encouraged to revisit and update their national designation of CEs for rare diseases and strengthen the organisation of national rare disease and specialised care networks - this should then translate to a more strategic engagement of national CEs with ERNs, via a limited number of full member HCPs.
6. ERNs should develop clear and transparent rules for patient engagement, adequately supporting the involvement of patient organisations and their representatives in the different ERN activities and fairly compensate patient representatives
7. The EU (EC) should provide coordination funding to coordinating HCPs but MS/EEA countries should provide a small amount of money to each national HCP/affiliated member within their national territory (providing they meet performance and impact indicators) to support their engagement in ERN activities
8. A special category of association or collaboration or affiliation should be created to allow formal collaboration and recognition of centres from countries outside of the EU MS/EEA; clear rules on shared activities (what is and is not permitted) should be created

SESSION 2 – INTEGRATING ERNS TO NATIONAL SYSTEMS AND FRAMEWORK

9. The European Commission should support Member States/EEA countries to implement the actions outlined in the ERN BoMs Statement on Integration of ERNs into national health systems, specifically by funding national multi-stakeholder workshops, with patient organisations, clinical leads and national authorities, to facilitate discussions and actions on integration into each of the national health systems.

10. The concept of a centre of expertise for rare diseases should be revisited at country level: countries should ensure they designate all such centres in a comprehensive and transparent way, and make the result of such a mapping and designation publicly available, demonstrating how ERN HCPs and 'affiliated' centres fit within wider national networks (where applicable). The *EUCERD Quality Criteria for Centres of Expertise for Rare Diseases* remain a robust resource here

11. To facilitate the referral of patients for ERN care, all Member States/EEA countries should endorse one centre as a 'National Coordination Hub', to function as a central referral management centre and gateway to accessing the specialist advice of the ERNs collectively: this centre should work in partnership with the national patient community, and build relationships with national professional societies and the research leads

12. National competent authorities should define national referral pathways for rare disease (or suspected rare disease) patients or those requiring a concentration of expertise, addressing transition from paediatric to adult care and containing clear guidance on how and when to seek referral to an ERN; ERNs should then compile and publish these pathways, explaining the process in each country and producing -with its patient advocates- patient-friendly information and advice on accessing specialist advice under an ERN.

13. ERNs should be supported to review and expand their disease-specific membership criteria, in collaboration with patients and professional associations, with an emphasis on the necessary multidisciplinary expertise: in this way, EU countries (perhaps even the global RD community) could make use of robust criteria by which to define expertise in given disease areas

14. ERNs and the BoMS should embrace a strategic mission of promoting more integrated care, encompassing integration of different medical specialities, but also of paramedical and social actors, in line with the *EUCERD Recommendations on Rare Disease European Reference Networks* and the *Commission Expert Group Recommendations to support the incorporation of rare diseases to social policies and services*

15. By 2025 a cross-ERN working group on integrated and holistic care should have been established in partnership with European Resources Centres for Rare Diseases, as a gateway to build joint

guidance on collaborative approaches for the provision of integrated and holistic care to people living with a rare disease: dedicated funding should be made available for broad stakeholder meetings and activities to advance this goal

16. ERNs should gather and create, in collaboration with patient organisations, resources which could support rare disease patients in receiving more integrated and more personalised care in their local environment: such resources should translate to heterogenous care and social settings, by focusing on clarifying and explaining the (often poorly-understood) needs of patients with complex conditions, and adaptations/approaches which could help

17. Renewing or updating national plans and strategies for rare diseases should remain a key priority for all countries - all such documents should stipulate the strategy to engage bidirectionally with ERNs, and support in this task should be provided by a group/body with a remit to encompass all RD topics, beyond ERNs alone

18. Each Member State/EEA should define a mechanism to disseminate and utilise the knowledge and evidence generated by the ERNs, to impact across the wider health and social systems: in particular, clinical practice guidelines/clinical decision support tools generated or endorsed by an ERN should be fully applied in all MS/EEA countries, and national committees and structures dedicated to rare disease or specialised healthcare should include some level of national ERN HCP representation

SESSION 3 – ROLE OF ERNS IN VIRTUAL CARE DELIVERY AND CROSS-BORDER HEALTHCARE

19. The CPMS should be fully compatible with any referring HCP systems, enabling automatic and two-way cross-talk with Electronic Health Records to populate and update records post case referral: data should be fully searchable, and cases accompanied by an appropriate PPRL (privacy preserving record linkage) solution to facilitate integration of ERNs

20. A dedicated study/project should be funded, to support countries in developing their EHRs and virtual care delivery services to best address the specificities of rare diseases and highly specialised healthcare, and promote interoperability - this could aim at wider national deployment of the CPMS or a system compatible with it, as the basis for virtual care provision for complex rare disease cases nationally

21. ERNs should develop strategies to minimise disparity in access to high quality healthcare between European regions - disease-related metrics should be agreed and monitored

22. An efficient project/tender should be funded to establish a pricing model to reimburse expert time spent on CPMS case review and propose alternatives for payment (e.g. a quid pro quo system, a straightforward billing of another MS/EEA country - perhaps with a differential GDP-based pricing scheme- a reduction in the workload of ERN HCP clinicians in lieu of payment for CPMS reviews, etc)

23. The social security regulation/ cross-border healthcare directive should be amended to allow for payment of time spent on cross-border virtual consultations performed through the CPMS, following a systematic national referral process

24. Countries should automatically authorise requests for treatments or therapies if deemed beneficial by an ERN panel through CPMS: the ERNs' expertise should hold more weight than national expert bodies who make such decisions as present

SESSION 4 – ERNS, RESEARCH, AND THE DATA ECOSYSTEM OF THE FUTURE

25. The future ERN health data strategy must be anchored to the wider European health data and IT ecosystem, driven by a concerted policy action of all the relevant DGs and aligned with national health data strategies from the majority of MS/EEA countries; in this context, ERNs should help to shape the future Health Code of Conduct for secondary use of data (addressing the need to make GDPR research-friendly)

26. The future ERN data strategy must be targeted towards all rare disease patients in Europe, and not only those attending ERN HCPs: opportunities must be created for patients to foster robust data partnerships, determine governance, and contribute/extract data to or from appropriate registries, care records and other relevant data sources.

27. By 2023, ERNs should develop a comprehensive data strategy and implementation plan envisaging the necessary activities across 6 action lines: architecture - cloud computing services and IT support for registries and other databases; data collection protocols; data curation services; data management tools (services and tools to search, access and share data, tools to manage own data); data analytics tools and services; and a data governance framework.

28. Data from hospital EHR (electronic health record) systems should be interoperable with the CPMS, and with ERNs' new epidemiological registries, allowing minimal data entry and maximum automation (with accompanying quality assurance)

29. Disease-specific registries (where positively evaluated by ERNs based on rigorous criteria OR

created anew by ERNs in future) should be interoperable with the new ERN registries and any robust national RD registries: these should all be connected (sustainably) to ERDRI* to provide a fully functioning registration ecosystem

30. ERNs should sit at the centre of all future efforts to refine and evolve all ontologies and standards for data collection and utilisation into a common data model, including efforts to facilitate extraction and mining from real-world data

31. ERNs should receive earmarked -and adequate- funding through European programmes to deliver clinical trials (involving centres inside or outside of the Networks, as required) and to research neglected topics including rehabilitative, holistic and social research

32. ERNs should be specifically and adequately funded to develop and conduct natural history (and where possible accompanying biomarker) studies, a minimum of 5 every 2 years, to build the knowledge base and capacity for clinical research in neglected diseases/areas lacking research

33. The Coordination and Support Action funded by the H2020 programme to support the creation of Clinical Research Networks (covering 4 domains: clinical research; data management; engagement and dissemination; and administrative support) should be supplemented by additional funding to deploy core services to become fully operational by 2025