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# EURORDIS

## Recommendations on the Integration of European Reference Networks (ERNs) into National Health Systems

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November 2018

ERNs create a clear governance structure for knowledge sharing and care coordination across the EU, but now we need to establish operating mechanisms that will govern their interaction with national health systems, including the organisational processes and structures to support the exchange of knowledge and information with national expert centres and networks and the rules for patient referral. The below Recommendations set out an overview of the most important actions needed to achieve an effective integration of ERNs into national health systems.

## Recommendations

- 1 [Create a good level of awareness at national level of the ERN model among all key stakeholders](#)
- 2 [Ensure political leadership and ownership of the ERN system at national level and generate sound evidence on the value of the ERN model](#)
- 3 [Review or adapt national policies on rare or low prevalence and complex conditions to address integration of ERNs into the national health systems](#)
- 4 [Member States to set up national networks of rare disease expert centres and the RD patient community to create national networks compatible with the ERN-disease groupings](#)
- 5 [Incorporate new Full Members and Affiliated Partners specifically to cover ERNs geographical and/or expertise gaps](#)
- 6 [Set up National Coordination Hubs and designate ERNs national focal points](#)
- 7 [Streamline the process to endorse healthcare providers that apply for full membership](#)
- 8 [Define and validate ERN referral pathway at national level to ensure timely access to ERNs advice and adequate management of case referrals](#)
- 9 [Establish a funding mechanism to finance ERNs virtual advice](#)
- 10 [Develop a mechanism to recognise and adopt at national level clinical guidelines reviewed or adopted by ERNs](#)

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# 1. Background

European Reference Networks (ERNs) are networks of healthcare professionals working in rare, low prevalence and complex diseases across Europe. They have been established to pool together the scarce and scattered medical expertise on rare and complex diseases throughout the European Union (EU) with the objective to provide a timely, accurate diagnosis and follow-up treatment for the patients affected by a rare or complex disease, wherever they live in the EU. The ultimate goal is to provide an equal access to specialised care, leaving no one rare disease patient behind.

On the 1<sup>st</sup> March 2017, the European Commission launched 24 ERNs, corresponding each to a broad disease grouping. These ERNs bring altogether nearly 1000 members, who are specialised healthcare providers on rare and complex diseases across EU countries, selected against stringent quality criteria by both national and EU competent health authorities.

The launch of these 24 ERNs is a landmark for the rare disease community since EURORDIS and its members have advocated for over 10 years for the establishment of European healthcare networks for rare diseases.

The integration of the European Reference Networks (ERNs) into national health systems refers to the set of policies, rules and procedures required to anchor the ERN system to the national level to ensure that all patients with a rare disease or complex condition across Europe can benefit from this model.

At present not all Member States of the European Union (MS) are represented in the different ERNs. This hinders access to ERNs for patients living in Member States where a given ERN does not have a full member. Likewise, this insufficient geographical coverage also represents a challenge to share and disseminate the knowledge and evidence generated by ERNs across all EU Member States.

Integration of ERNs into national health systems is required to overcome this accessibility problem and to facilitate knowledge and expertise to drill down into all national health systems. Only then will the ERNs' system deliver on its full potential and benefit patients equally, regardless of where they live.

Certainly, the ERN "enlargement" agenda, through the addition of new full members and the designation of Affiliated Partners (first wave will be limited to Associated National Centres and National Coordination Hubs), will contribute to bridge the gap between ERNs and national health systems. **However, more is needed to connect the ERN system into the national healthcare infrastructures. This is an ambitious and complex process with major technical as well as budgetary implications, that requires at the very least:**

- Member States to define a clear framework, including referral pathways, organisational processes, policies and infrastructure to link their own national healthcare systems with ERNs;
- ERNs to develop their integration strategies for affiliated partners as well as exploring other innovative ways to cooperate with other centres of expertise and networks at national and international level;
- Having in place the adequate infrastructure and organisational processes to allow knowledge and expertise to be accumulated and shared beyond ERNs- reaching out the national and regional and local levels.

It is also important to underline that the ERN system is the result of the multi-stakeholder collaboration and work done over more than a decade:

- 2004 - 2009 (Rare Diseases Task Force and High Level Group on Health Services and Medical Care);
- 2010 - March 2017 (EU Committee of Experts on Rare Diseases; Commission Expert Group on Rare Diseases; EUCERD Joint Action and RD-ACTION –Joint Actions on Rare Diseases; Working Group

Member States on ERNs; EC Delegated and Implementing Decisions, Consortium for the Application and Assessment Process of ERNs: PACE-ERN; establishment of the Board of Member States),

- March 2017- ongoing (Official launch of ERNs; Working Group of ERN Coordinators; reinforcement of DG Santé Unit responsible for ERN; deployment phase).

Although conceptually initiated years ago and developed over the past decade, the ERN system was formally established after the adoption of the 24 National Plans on Rare Diseases (following the Council Recommendation on Action in the field of Rare Diseases of June 2009<sup>1</sup>, these plans were gradually adopted between 2013-2018). One of the core elements of these plans is the designation of centres of expertise and the criteria required to be formally recognised as such. Many National Plans for Rare Diseases include measures to link up centres of expertise with ERNs; however, these measures need to be adjusted with the most recent rules and procedures for establishing and developing ERNs. Indeed, **the ERN system now provides a set of operational criteria to qualify for full membership. In order to overcome discrepancies within countries with different quality standards and a mismatch between the national centres of expertise and the centres that have been endorsed as full members of a given ERN, it is important to ensure the greatest possible alignment of MS accreditation criteria for centres of expertise with the operational criteria for ERN full members.** At the same time, an effective integration of ERNs into national health systems may require adjustments in the RD National Plans to address the technical capacities as well as the organisational and legal reforms required to anchor this new structure in each national health system.

In October 2017, the ERN Board of Member States (BoMS) established a Working Group on Integration of the ERN composed of Member States representatives, ERN Coordinators and Commission representatives. This Group will provide guidance to the Member States on the following strategic issues<sup>2</sup>:

- Establishing **rules for the referral of patients** to the ERNs, having first defined patient pathways in the national healthcare systems;
- Agreeing on the form of **support to be provided by the Member States to the Coordinators and the ERN members**;
- Facilitating **cost reimbursement in case of patient mobility triggered by ERN advice**;
- Procedures for endorsement of healthcare providers becoming ERN Members;
- **Identification of the changes required in national policy and legal frameworks** needed for the integration of the ERNs into the national health systems;
- **Provision of information on ERNs at Member State level.**

The mandate of the group reflects that, at this stage, the primary focus of Member States is to enable the referral of cases for ERN virtual consultations and of patients for cross-border healthcare. ERNs create a clear governance structure for knowledge sharing and care coordination across the EU, but the system will fail to reach its full potential if it does not benefit the wider patient community, beyond the small number of cases that will be reviewed each year through the ERNs Clinical Patient Management System. Therefore, it is equally important to establish the organisational processes and structures to support the exchange of knowledge and information with national expert centres and networks, as well as to share developments on data collection and data exchange to align the future ERN health data ecosystem with the national digital health initiatives.

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<sup>1</sup> Council Recommendation (2009/C 151/02) of 8 June on an action in the field of rare diseases  
<http://eurlex.europa.eu/LexUriServ/LexUriServ.do?uri=OJ:C:2009:151:0007:0010:EN:PDF>

<sup>2</sup> [Minutes of the Board of Member States meeting, 10 October 2017](#)

## 2. Summary of Recommendations

### A. Recommendations to create an enabling environment for the integration of ERNs into national health systems

1	<p>Create a good level of awareness at national level of the ERN model among all key stakeholders</p> <ul style="list-style-type: none"><li>All stakeholders involved in ERNs should contribute to raise national awareness. There should be a more decided effort by MS to contribute to this effort, since they are in the best position to reach out to the relevant stakeholders at national and local level to explain the benefits of the ERN model and address any concerns. Clinicians and patient advocates already involved in the ERNs could help on this effort at national level.</li></ul>
2	<p>Ensure political leadership and ownership of the ERN system at national level and generate sound evidence on the value of the ERN model</p> <ul style="list-style-type: none"><li>The long-term sustainability of ERNs requires all MS to take ownership of the ERN model at the highest political level and across different government portfolios. Adequate funding, appropriate resources and long-term financial planning is required if ERNs are to function as permanent structures and ensure the continuity of their operations.</li><li>We encourage ERNs, the European Commission and the BoMS to prioritise ERNs' performance measurement and commit the resources required to implement the ERNs continuous monitoring framework and ensure a good measurement of the benefits for the wider patient population. Also we invite them to disseminate more widely the results of the ERNs periodical self-assessment exercise and the results of their activities.</li><li>All MS should identify and define their demand for ERN services. This kind of planning, will allow ERNs to plan ahead, adapt their activities and organise their resources to better respond to the national health systems' needs. MS with decentralized health systems, should establish a strong coordination mechanism with the regional health authorities to capture their needs.</li><li>To facilitate coordination on all aspects linked to ERNs we call on MS to set up a national ERN coordination group composed by all relevant stakeholders including, ePAG advocates, RD National Alliances, clinicians, researchers, hospital managers, and health and social care policy makers at national and regional level.</li></ul>
3	<p>Review or adapt national policies on rare<sup>3</sup> or low prevalence and complex conditions to address integration of ERNs into the national health systems</p> <ul style="list-style-type: none"><li>MS will need to either review or adapt their national strategies on rare, low prevalence and complex conditions to address the technical capacities, as well as the organisational and legal reforms required to anchor ERNs into their national health and social care systems and facilitate broad dissemination of knowledge and expertise on all matters related to the provision of holistic-integrated care for people living with these conditions.</li><li>We encourage MS that still lack an overall national strategy on rare, low prevalence and complex conditions to take immediate action to adopt it.</li><li>With the ERN system now providing a set of operational criteria to qualify for full membership, the way forward is that MS align their accreditation criteria for centres of expertise with the operational criteria for ERN full members. Also it is important to underline that accreditation of centres of expertise, as well as participation in ERNs, should be a dynamic and evolving process.</li></ul>

<sup>3</sup> In these Recommendations the term rare diseases includes also rare cancers

4	<p>Member States to set up national networks of RD expert centres and the RD patient community to create national networks compatible with the ERN-disease groupings</p> <ul style="list-style-type: none"> <li>MS should explore the feasibility of creating national rare disease reference networks, integrate them into the respective RD National Plans or Strategies and engage with clinicians and patients to set them up and drive their implementation.</li> <li>The rare disease patient community at national level should consider re-organising their network activities around a structure compatible with ERN-disease groupings. This would allow them to work across disease areas that share similarities and facilitate the cooperation with the 24 European Patient Advocacy Groups.</li> </ul>
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## B. Recommendations to implement the ERN enlargement agenda and create mechanisms to enable integration into national health systems

5	<p>Incorporate new Full Members and Affiliated Partners specifically to cover ERNs geographical and/or expertise gaps</p> <ul style="list-style-type: none"> <li>Healthcare providers from underrepresented MS should join the ERNs, including: Austria, Croatia, Cyprus, Estonia, Greece, Latvia, Lithuania, Malta, Slovak Republic, Slovenia, Spain and Luxembourg.</li> <li>We encourage all ERNs to expand their disease coverage within their disease grouping according to the level of maturity of the clinical network and following a stepwise approach to leave no patient behind.</li> <li>The RD patient community, RD National Alliances and the ePAG community, should be formally consulted in any future ERN enlargement process.</li> </ul>
6	<p>Set up National Coordination Hubs and each ERN to designate national focal points</p> <ul style="list-style-type: none"> <li>MS should set up ERN National Coordination Hubs to link with the ERNs that are not represented in their territories. These Hubs should play a dual role as an entry point for ERN advice and as a knowledge management hub.</li> <li>We encourage ERN Coordinators and the BoMS to work together to develop the figure of "ERN national focal point" to formally designate one healthcare provider in each MS that would be the entry point and knowledge hub for that ERN at national level.</li> <li>In both cases, MS with a decentralised health system, should put in place a strong coordination mechanism on ERNs with their regional health authorities.</li> </ul>
7	<p>Streamline the process to endorse healthcare providers that apply for full membership</p> <ul style="list-style-type: none"> <li>In the light of the new call for full members, we encourage MS to share good practices to streamline their endorsement processes while ensuring a good level of transparency in their decision-making, to guarantee that the quality of the process and the bar applied to healthcare providers is the same across all countries. They will also need to plan ahead and allocate extra resources, if needed, to perform the validation of HCPs in a smooth and rigorous way.</li> </ul>
8	<p>Define and validate ERN referral pathway at national level to ensure timely access to ERNs advice and adequate management of case referrals</p> <ul style="list-style-type: none"> <li>The national ERN referral pathways should be developed in accordance with national legislation, with the involvement of the local clinicians and patient community and also in consultation with ERN Coordinators.</li> </ul>
9	<p>Establish a funding mechanism to finance ERNs virtual advice</p> <ul style="list-style-type: none"> <li>An adequate funding mechanism to finance the ERNs virtual advice is critical to maintain the provision of this service and eventually scale it up to respond to an increase number of referrals for ERN advice, once ERNs are effectively integrated into national health systems.</li> </ul>

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Develop a mechanism to recognise and adopt at national level clinical guidelines reviewed or adopted by ERNs

- MS, through the BoMS, should define common process to endorse ERNs guidelines. This process should have a central point for review and approval of ERN guidelines based on robust assessment of the guidelines and recommendation for adoption in Member States.
- MS are recommended to define their protocol for assessing and adopting ERN guidelines into their national health system, including the legal status of ERN guidelines and the national body responsible for assessing the guidelines for adoption.
- MS are encouraged to include their national patient organisations and ePAG advocates in their Guidelines Advisory Committee as well as in their protocol for the assessment and adoption of ERN guidelines into their national health system.



## 3. Recommendations

### Create an Enabling Environment

#### 1. Create a good level of awareness at national level of the ERN model among all key stakeholders

ERNs were launched in 2017, but there is still a low level of awareness of the ERN model in most Member States. Key stakeholders at national level including **national and regional policy makers, hospital managers, healthcare professionals, researchers and the patient community** are not yet fully aware of what is the rationale behind the ERN system; how it fits in the wider context of the European policy on cross-border healthcare and the national RD strategies; what is the objective; what activities are ERNs undertaking; what disease areas fall under each ERN and what is their governance structure.

**All stakeholders involved in ERNs should contribute to raise national awareness.** Patients and clinicians are already contributing to this effort through their networks and organisations; the European Commission has launched an external communications campaign on ERNs (2018-2019) targeted at healthcare professionals, hospital managers and patients<sup>4</sup>.

There should be a **more decided effort by Member States** to contribute to this effort. MS should actively support the European Commission's communication campaign on ERNs and relay information since they are in the best position to reach out to the relevant stakeholders at national and local level **to explain the benefits of the ERN model and address any concerns**. Clinicians and patient advocates already involved in the ERNs could help on this effort at national level.

Some of the actions by MS could include:

- Supporting the communication activities driven by other stakeholders at national level (National Alliances of Rare Diseases, Patient Organisations, patients, clinicians and researchers)
- Engaging with the national RD patient and scientific community to inform them about the services and possibilities offered by ERNs,
- Engaging with hospital managers to explain how they can benefit from ERNs and how they can provide support to their local coordinators and members.
- Explaining regional health authorities what is the value of the ERN model, how they fit into the national rare disease strategy and how they can contribute to deliver more efficient and better quality care.
- Addressing any concerns that regional health authorities or hospitals managers might have with regards to the budgetary impact linked to the participation of hospitals as full members or affiliated partners or to reimbursing cross-border healthcare services in case patient mobility is triggered by ERN advice.
- Supporting the translation of relevant documents. A substantial amount of the information that is being generated by the ERNs is only available in English. Translation of relevant documents and other materials, not only the official ones produced by the European Commission, to other languages is critical to raise awareness on the value of ERNs.

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<sup>4</sup> Communication materials are available in this website [https://ec.europa.eu/health/ern\\_en](https://ec.europa.eu/health/ern_en), under the heading Related Information

## 2. Ensure political leadership and ownership of the ERN system at national level and generate sound evidence on the value of the ERN model

The ERN model is still in an early stage of deployment, but it is already clear that ensuring its long-term sustainability will require support from the Member States at the highest political level. The launch of the 24 ERNs in 2017 was a strong signal for the rare disease community, but it remains unclear yet how and to what extent Member States will support this new model on the long run and how it will be funded.

Be it through national or EU funding mechanisms, MS have the last word as to the level of resources that will be allocated to ERNs. Adequate funding is required to support the coordination of the networks, sharing of knowledge and information, development of clinical guidelines, registries, meaningful patient engagement in ERNs, training and educational activities, virtual consultations as well to finance the digital technologies required to support all these networks' activities. On top of this, integration of ERNs could eventually also trigger additional costs for each Member State, as they will need to put in place the necessary infrastructure and resources to effectively bridge the gap between the national health system and each ERN.

Today ERNs lack a long-term financial planning. Without careful financial planning, relying on different sources of funding poses the risk of managing the different activities of the ERNs as temporary stand-alone projects, rather than as functions of a permanent structure. The challenge for the ERNs Coordinators will be to manage a fragmented funding environment that adds bureaucracy, is time-consuming and may prevent ERNs to function as permanent structures.

Financial planning for ERNs is also essential at national level, as MS need to define the resource allocation for HCPs participating to ERNs in their countries by tapping into both national *and* EU financial resources. There are various EU funding mechanisms that could be used to support the work of these Networks in the next long-term EU budget 2021-2027, including the European Structural Funds, notably ERDF and ESF+, the Digital Europe programme, Horizon Europe, InvestEU Programme or European Investment Bank loans. MS need to plan well in advance how they would like to use each of these instruments to fund ERNs operations and infrastructure and what additional national funds can be mobilised to support the healthcare providers that are participating in the Networks.

In particular, Structural Funds (SF) have a role to play in the integration of ERNs into national healthcare systems (e.g. support of HCPs, national networks and/or centres of expertise; funding training of professionals; support for the deployment of digital infrastructure and services and interoperability). To tap into this potential, it is crucial that, for the next SF programming period, MS identify these interventions as priorities into Thematic Objectives and include them in their Operational Programmes.

ERNs have the potential to improve quality of care and health outcomes, develop scientific excellence and bring advances in research and expertise on complex and rare diseases, helping MS to strengthen their own health systems' diagnostic and treatment capacities. Providing evidence on the value of ERNs for the wider patient community, measuring results and evaluating their performance is critical to mobilise funds, but also to ensure that the system remains in place regardless of political changes at EU or national level.

The long-term sustainability of ERNs requires all **MS to take ownership of the ERN model at the highest political level and across different government portfolios** including finance, health and social care, research and education. **Adequate funding, appropriate resources and long-term financial planning is required if ERNs are to function as permanent structures and ensure the continuity of their operations.**

At the same time, we need to build compelling evidence on the value of the model. In order to build such an essential evidence base, we encourage ERNs, the **European Commission and the Board of Member States**

**(BoMS) to prioritise ERNs' performance measurement and commit the resources required to implement the ERNs continuous monitoring framework** and ensure a good measurement of the benefits for the wider patient population. Also we invite them to disseminate more widely the results of the ERNs periodical self-assessment exercise and of their activities.

On a more operational level, ownership of the ERN model by MS would be secured if this new structure is able to effectively respond to the needs of national health systems. **All MS should identify and define their demand for ERN services** (training, expertise, implementation of clinical practice guidelines, recommendations, virtual advice, etc). This kind of planning, will allow ERNs to plan ahead, adapt their activities and organise their resources to better respond to the national health systems' needs. MS with decentralized health systems, should establish a strong coordination mechanism with the regional health authorities to capture their needs.

To facilitate coordination on all aspects linked to ERNs, we call on MS to set up a **national ERN coordination group** composed by all relevant stakeholders including, RD ePAG advocates, RD National Alliances, clinicians, researchers, hospital managers, and health and social care policy makers at national and regional level.

### 3. Review or adapt national policies on rare, low prevalence and complex conditions to address integration of ERNs into the national health systems

Beyond funding, unleashing the full potential of the ERNs system requires a **concerted inter-sectoral response** at national level.

ERNs will pool and develop good practices and standards of care; define integrated care pathways for rare or low prevalence and complex diseases; provide guidance on how to best deliver multidisciplinary care; develop new knowledge on treatments and clinical guidelines; undertake research; develop registries as well as training and education opportunities. All this information and knowledge should then be captured, shared and assimilated at national level, contributing to increase the national health systems capacities for the diagnosis and treatment of rare or low prevalence and complex conditions. This will not happen overnight; it requires careful planning and a strategy at national level to put in place the technical capacities, infrastructure and organisational arrangements for effective knowledge management.

At this stage, the focus of ERNs is primarily a medical one but, over time, we expect that they will evolve to incorporate social and care aspects. Holistic integrated care is central to improve the quality of life of people living with rare or low prevalence and complex diseases. ERNs could play a key role in identifying, developing and/or spread good practices and standards of care (e.g. defining holistic pathways and elaborating descriptions of patients' needs and of "ideal" multidisciplinary teams; share good practices used by HCPs; engage in tertiary prevention by providing guidance).

In order to implement future ERN recommendations and good practices on integrated care, MS should already adopt the organisational and technical measures required to enable integrated care pathways that will allow the exchange of information and the coordination between health, social and other support services located at national, regional and local levels.

MS will need to either **review or adapt their national strategies on rare, low prevalence and complex conditions to address the technical capacities, as well as the organisational and legal reforms required to anchor ERNs into their national health and social care systems** and facilitate broad dissemination of knowledge and expertise on all matters related to the provision of multidisciplinary and integrated care for people living with these conditions.

We encourage MS that still lack a national strategy on rare, low prevalence and complex conditions to take immediate action to adopt it.

Changes may also be required at the level of the establishment of clear criteria and a transparent process to designate centres of expertise at national level and of the identification of patient pathways.

As laid down in the 2009 Council Recommendation<sup>5</sup>, the identification by Member States of “appropriate centres of expertise throughout their national territory by the end of 2013” and the support to their creation, is a core element of Rare Diseases National Plans and Strategies. In 2011, the EUCERD recommended quality criteria for RD centres of expertise<sup>6</sup>, “intended to help MS in their reflections or policy developments concerning national plans and strategies for rare diseases when addressing the issue of organisation of healthcare pathways at national and European level”. These Recommendations provided further details for countries to designate their centres and to ensure a certain degree of uniformity across the EU. Still, despite these Recommendations and 24 of RD Plans or Strategies adopted as of September 2018, not all EU countries have established criteria to formally identify such national expertise and, when it happens, discrepancies between countries are still evident.

It should also be noted that in some countries that lack a formal designation process for centres of expertise, the concern is that endorsement of HCPs to join ERNs has replaced the essential national effort to identify and map expertise in the country and, similarly, to establish patients’ healthcare pathways, which can be complemented but not replaced by European Networks.

In line with EU policies and recommendations, there is a need for MS to adjust and harmonise their process and criteria for designating their national centres of expertise on rare or low prevalence and complex conditions, thus recognising their clinical excellence. With the ERN system now providing a set of operational criteria to qualify for full membership which has incorporated the EUCERD criteria and developed them further, **the way forward is that MS align their accreditation criteria for centres of expertise with the operational criteria for ERN full members. This alignment would overcome the risk of having different quality standards and a mismatch between the national centres of expertise and the centres that have been endorsed as full members of a given ERN.** Also, it is important to underline that **accreditation of centres of expertise, as well as participation in ERNs, should be a dynamic and evolving process.**

MS are recommended to review, and if needed, adapt their national plan or strategy for rare diseases, with a view to formally reflect this way forward and implement a harmonised process that will optimise the mapping of national expertise, further facilitate networking of medical experts at European level while at the same time, ensuring relevant referrals to these ERNs and thus, their integration into national healthcare systems.

#### 4. Member States to set up national networks of RD expert centres and the RD patient community to create national networks compatible with the ERN-disease groupings

The creation of networks of centres of expertise should be also pursued at national level, at least in countries whose size substantiates this organisational approach. In fact, these networks already existed in some countries before ERNs were launched (e.g. the French national RD networks – filières- or the Italian network for rare cancers). Another option could be leveraging on the existing infrastructure of scientific societies.

Having a well-organised network-like national approach to rare disease diagnosis, treatment and care is essential for the proper functioning of healthcare pathways for people living with RD, but also for the efficient operations of ERNs (which should complement but never unnecessarily replace national pathways for patients).

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<sup>5</sup> Council Recommendation (2009/C 151/02) of 8 June on an action in the field of rare diseases  
<http://eurlex.europa.eu/LexUriServ/LexUriServ.do?uri=OJ:C:2009:151:0007:0010:EN:PDF>

<sup>6</sup> [http://www.eucerd.eu/?post\\_type=document&p=1224](http://www.eucerd.eu/?post_type=document&p=1224)

National networks that replicate the ERN structure at national level would simplify the integration of ERNs into national health systems, optimising and facilitating the consistency of patient referrals to ERNs and the flow of knowledge between network structures. Also, having national RD reference networks especially in the bigger MS would help to achieve an adequate geographic balance in ERNs and avoid the overrepresentation of big MS.

National networks of centres of expertise would also provide a structure for patients and clinicians in each Member State to work more closely across administrative boundaries and areas of expertise. Ultimately, the same rationale that underpins the ERN system is also valid at national level: pooling together resources and expertise on rare diseases at national level, could contribute to improve the delivery of healthcare and reduce the disparities in access to care for RD patients that we see in some Member States.

Likewise, the rare disease patient community at national level would also benefit from re-organising their network activities around similar disease groupings. This would facilitate the cooperation with the 24 European Patient Advocacy Groups and allow them to work across disease areas that share some commonalities.

Member States, where this approach is not yet implemented, should **explore the feasibility of creating national rare disease reference networks**, integrate them into the respective National Plans or Strategies and engage with clinicians and patients to set them up and drive their implementation.

The **rare disease patient community at national level** should consider re-organising their **network activities around a structure compatible with ERN-disease groupings**. This would allow them to work across disease areas that share similarities and facilitate the cooperation with the 24 European Patient Advocacy Groups.

## Implement the ERN enlargement agenda and create the mechanisms to enable integration into national health systems

### 5. Incorporate new Full Members and Affiliated Partners specifically to cover ERNs geographical and/or expertise gaps

The ERN enlargement agenda, through the addition of new full members and the designation of Affiliated Partners, will contribute to bridge the gap between ERNs and national health systems. It is important to underline that ERNs membership should be a dynamic and evolving process, reflecting the evolution of the centres in their capacities and performance, experts' mobility, re-organisation of the national health systems and healthcare providers' mergers.

The affiliation process includes the following steps: (i) the identification of affiliated partners (by MS); (ii) the development of an affiliation strategy (by ERNs and approved by BoMS), and (iii) the integration of selected Associated National centres into the respective ERNs. At the same time, the European Commission may launch calls for healthcare providers to join the ERNs as new full members. **Any enlargement process needs to be adequately managed to avoid disrupting the work of the ERNs, especially at this early stage.**

**In any case, the enlargement of ERNs should always be oriented towards covering the geographic and expertise gaps.**

We encourage Member States to ensure that the ERNs enlargement process will specifically be targeted at **reducing the expertise and geographic gaps.**

Healthcare providers from underrepresented Member States should join the ERNs, including: **Austria, Croatia, Cyprus, Estonia, Greece, Latvia, Lithuania, Malta, Slovak Republic, Slovenia, Spain and Luxembourg.**

With regards to the expertise gap, we **encourage all ERNs to expand their disease coverage within their disease grouping** according to the level of maturity of the clinical network and following a stepwise approach to leave no patient behind.

The **RD patient community, RD National Alliances and the ePAG community, should be formally consulted in any future ERN enlargement process.** MS and ERN Coordinators should engage with them to identify the different expertise and geographic gaps. Patients are in a privileged position to contribute to this mapping process through their own experience and networks.

## 6. Set up ERN National Coordination Hubs and designate ERNs national focal points

The full integration of ERNs into national health systems requires having a dual and complementary outreach strategy by MS and ERNs:

1. Not all Member States will be able to have HCPs as full members or Associated National Centres in all 24 ERNs. Having a national ERN Coordination Hub could help them establish an interface with the ERNs that are not represented in their territories. Regardless of the type of organisation that is designated as a National Coordination Hub, it should play a dual role as an entry point for ERN advice and as a knowledge management hub to disseminate and share information and knowledge generated by those ERNs that have no presence in their territory<sup>7</sup>.
2. At the same time, it is equally important that ERNs establish mechanisms to cooperate and reach out to national healthcare providers and national rare disease networks. Each ERN should formally designate in each MS where they have a member, a national focal point to be the entry point and formal bridge between the ERN and the national health system.

MS should set up ERN National Coordination Hubs to link with the ERNs that are not represented in their territories. These **Hubs should play a dual role as an entry point for ERN advice and as a knowledge management hub to disseminate and share information and knowledge** generated and pooled by those ERNs that have no presence in their territory.

We encourage the ERN Coordinators and the BoMS to work together, in close collaboration with the ePAG advocates, **to develop the figure of an “ERN national focal point”** and formally designate one healthcare provider in each MS that would be the entry point and knowledge hub for that ERN at national level.

In both cases, MS with a decentralised health system, should put in place a strong coordination mechanism with the regional health authorities on ERNs.

## 7. Streamline the process to endorse healthcare providers that apply for full membership

In the lead-up to the establishment of ERNs, each country was responsible for defining its own procedure by which to endorse their HCPs to participate to ERNs. The national assessment and endorsement of HCPs applications submitted in the first wave, showed disparities across Member States. This process was too slow in certain Member States, which led to a very small number of endorsed HCPs, and in some MS the patient community raised concerns regarding the quality of the process. Given that healthcare providers are assessed against the same operational criteria, the endorsement process for HCPs should not differ greatly from one country to the other.

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<sup>7</sup> EURORDIS is working with the National Alliances of 8 Member States to define the functions and requirements for ERN National Coordination Hubs and will engage with the Board of Member States and ERNs on this effort.

In the light of the new call for full members, we encourage MS to share good practices to streamline and their endorsement processes while ensuring a good level of transparency in their decision-making, to guarantee that the quality of the process and the bar applied to healthcare providers is the same across all countries. They will also need to plan ahead and allocate extra resources, if needed, to perform the validation of HCPs in a smooth and rigorous way.

## 8. Define and validate ERN case referral pathway at national level to ensure timely access to ERNs advice and adequate management of case referrals

Individual patients do not have direct access to ERNs. However, their local specialist can refer the case to the relevant ERN member, or in the future to the relevant Associated National Centre, so that they can refer the case for virtual consultation within their ERN.

**National ERN case referral pathways should be developed in accordance with national legislation, with the involvement of the local clinicians and patient community and also in consultation with ERN Coordinators.** They should be supported by a series of standard operating procedures (setting roles, escalation processes, escalation for resolution of delays and other issues, etc.) which can be adapted and amended to respond to changes in local or national policies. In some cases, the referring clinician will also need translation support.

An effective ERN case referral pathway should be based on the following guiding principles<sup>8</sup>:

- Ensure that it will deliver a short wait, and clearly describe what should happen, in what order and when.
- Ensure that the patient pathway at national level has been completed and that national RD diagnostic and treatment capacities have been exhausted before the case is referred.
- Achieve an adequate balance between local demand for ERN case referrals and ERN capacity.
- Refer patients in order of clinical priority; and for those with the same clinical priority, in the order they were added to the waiting list.
- Actively manage patients along the pathway for their condition and the important milestones.
- Clinicians and patients are aware of the ERN referral process and this is applied consistently throughout the national health system.
- Translation support is provided at national level when needed to refer the case or translate the results of the ERN advice.

At a minimum, the description of the ERN case referral pathway will need to include the following elements:

1. **Referral management process.** Identify tasks end to end, define roles and responsibilities of the referring clinician and the Coordination Hub, milestones, estimate duration, management of incidences and delays.
2. **Administrative criteria (administrative triage).** This includes identifying whether and in what cases local healthcare professionals can refer cases to the ERN National Coordination Hub.
3. **Clinical review (clinical triage - gatekeeping).** Identify the clinical assessment findings that should prompt the referral of cases for ERN advice. Establish a panel of local clinicians to provide clinical reviews as a two-way process with the referring clinician, to ensure that only the cases that cannot be diagnosed or adequately treated at national level are referred for ERN advice.
4. **Liability.** Make clear where clinical responsibility lies for the patient throughout the referral journey.

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<sup>8</sup> [Elective care guide Referral to treatment pathways: A guide to managing efficient elective care, third edition, NHS. May 2017 \(adapted\)](#)

5. **Management of cases post-ERN advice along their outpatient and inpatient pathways.** This includes guiding patients, facilitating booking for appointments for further diagnostic tests or exploratory examinations and offering them the option of grouping consultations or tests on the same day and organising the appointments.

## 9. Establish a funding mechanism to finance ERNs virtual advice

In order for the ERNs to be fully incorporated on a national level, there needs to be incentives and resources for clinicians and researchers to allocate the appropriate amount of time to ERN-related work.

Eventually, the integration of ERNs into national health systems, will lead to an increase in the number of cases that will be referred to the ERNs for virtual advice. However, today ERN virtual advice delivered through the clinical patient management system is being provided by clinicians on a voluntary basis.

An **adequate funding mechanism to finance the ERNs virtual advice is critical to maintain the provision of this service** and eventually scale it up to respond to an increase number of referrals for ERN advice, once ERNs are effectively integrated into national health systems.

## 10. Develop a mechanism to recognise and adopt at national level clinical guidelines reviewed or adopted by ERNs

Knowledge and expertise of rare diseases is at best limited in every country, with many rare diseases being misdiagnosed resulting in multiple costly tests and inappropriate treatments. European Reference Networks offer an opportunity to draw on the collective knowledge and expertise from across Europe and enable the expertise to travel instead of the patient. Clinical guidelines are a major vehicle for this scarce knowledge to be communicated and utilised locally in each Member State, guiding diagnosis and treatment, reducing the burden of rare diseases on both patients and health systems.

Clinical guidelines, treatment protocols and care standards developed in ERNs will be one of the key networking activities that offer all countries the opportunity to share the latest thinking and evidence base, enabling Member States to tackle the public health needs of rare diseases, offering improved access to high quality diagnosis, care and treatment. These guidelines, protocols and standards are developed by the recognised experts that have been endorsed by Member States. The recognition and implementation of these guidelines in each, and every, Member State is critical to deliver on the ambition of the ERNs to share, care and cure.

Member States, through the ERN Board of Member States, are asked to **define common process to endorse ERNs guidelines**. This process should have a central point for review and approval of ERN guidelines based on robust assessment of the guidelines and recommendation for adoption in Member States.

Member States are recommended to **define their protocol for assessing and adopting ERN guidelines into their national health systems**, including the legal status of ERN guidelines and the national body responsible for assessing the guidelines for adoption.

Member States are encouraged to **include their national patient organisations and ePAG advocates in their Guidelines Advisory Committee as well as in their protocol for the assessment and adoption of ERN guidelines** into their national health system.





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