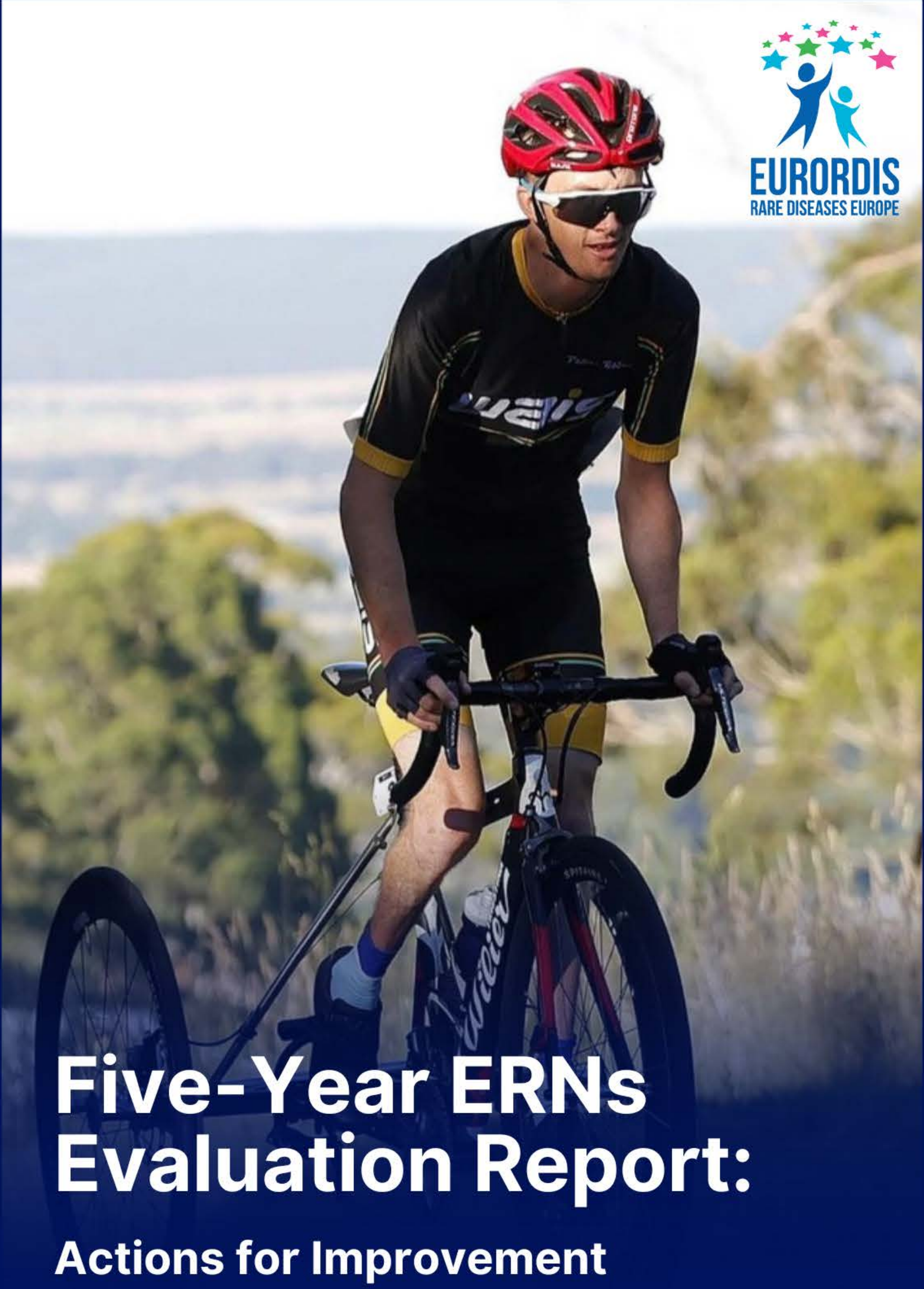

Five-Year ERNs Evaluation Report:

Actions for Improvement

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9 May 2025

Front cover photo: ‘*What I do best*’, Andrew Bannister, who lives with Periventricular Nodular Heterotopia, Polymicrogyria and Cerebellar Hypoplasia.

1. SUMMARY OF EVALUATION RESULTS

The Commission launched the first evaluation of the ERNs in December 2022, 5 years after the launch of the ERNs, to assess:

- the fulfilment of the criteria and conditions in Delegated Decision 2014/286/EU
- the accomplishment of the objectives of Article 12(2) of Directive 2011/24/EU
- the network's outcomes and performance and each member's contribution

All 24 ERNs and 836 healthcare providers (HCPs) from 24 EU Member States and Norway participated in this evaluation process. The evaluation included self-evaluations, document reviews, ERN interviews, on-site HCP audits and stakeholder interviews. It was performed by an Independent Evaluation Body (IEB) and led to a final report published on February 2024.

Overall, the evaluation concluded that the ERN ecosystem is functioning well. 100% of ERNs and 87.68% of HCPs achieved a satisfactory result. Of the 84 HCPs with unsatisfactory results, 72 have submitted an improvement plan and have 1 year to implement the proposed actions. The 72 will go through a re-evaluation process in 2025. Only 31 HCPs of the 836 under evaluation had their membership terminated.

The evaluation assessed the structure of the networks (i.e. geographic coverage), their activities, level of maturity, and impact.

- **Structure:** There is broad geographic coverage but regional disparities (East-West) remain. The evaluation found a notable lack of uniformity in the distribution of HCPs across Europe, with the Western European region having a higher concentration of HCPs.
- **Activity:** ERNs have successfully developed most planned activities.
- **Maturity**: ERNs' system objectives are effectively pursued but there is a need to support and motivate HCPs for their continuous involvement in the ERNs.
- **Impact:** ERNs have increased the visibility of rare and complex conditions and have allowed extensive exchange of specialised knowledge. However, there is a need to create and use tools to analyse impact.

The evaluation report identifies Networking and Dissemination, and Education and Training as the strongest activity areas for the ERNs.

- **Networking and Dissemination**: ERNs have developed a clear communication strategy to increase visibility and raise awareness of their activities, best practices, and expertise. The networks are effective in tailoring messages to specific audiences, ensuring that accurate and relevant information reaches healthcare professionals, including clinicians, referring physicians, and specialists.

- **Education, Training, and Professional Development**: ERNs have identified the education and training needs within the rare disease care ecosystem and have initiated various activities to address them. These initiatives aim to involve a broad range of professionals, both within and outside the ERN network, in continuous education and professional development.
- **Governance and Coordination**: ERNs have established clear governance frameworks that define their objectives, activities, and the relationships between HCPs and the networks. These structures also involve patient organisations, with some having representation in the ERN governing bodies, allowing for patient input in strategy and activity development.
- **Contribution to Research**: ERNs play a vital role in sharing data and creating collaborative registries and databases at the EU level. Financial support from the EU has facilitated the development of comprehensive rare disease registries.

2. OPPORTUNITIES FOR IMPROVEMENT

The ERNs 5-year evaluation report identifies opportunities for improvement in five different areas. Drawing on the analysis of some of these recommendations, this paper proposes further action to:

1. Formalise and implement patient partnership in the ERNs
2. Diversify sources of funding and review the funding system
3. Intensify the focus on integration
4. Measuring outcomes of care and clinical performance, instead of outputs
5. Developing a quality improvement system

1. FORMALISE AND IMPLEMENT PATIENT PARTNERSHIP IN THE ERNS

IEB Evaluation Report. *According to the report, roughly 80% of ERNs demonstrate a firm dedication to involving patient representatives.*

EURORDIS' vision is that by 2027, after 10 years of operations, all 24 ERNs should have **implemented** their own patient partnership model, based on healthy and systematic collaboration, that is well-supported and assessed over time to celebrate achievements and to make the necessary adjustments. In this sense, EURORDIS considers that implementation of patient partnership has reached a stage of maturity in an ERN when:

- A commitment to **patient partnership values and principles** are found in key organisational documents (e.g., ERN mission and vision, strategy, etc.).
- Collaboration is **systematic** throughout the Network, not only carried out by a few health professionals and ePAG leads in specific projects or at certain stages of a project.
- The partnership agenda is **driven by both parties**. Projects and activities that require partnering are being initiated either by patient representatives or health professionals, with equal opportunities for being approved and funded by the ERN.
- Collaboration is **well-supported** through the structure of the ERN, its organisational processes, and a project manager within the Coordination team that has the skills, experience and knowledge of tools and methods to facilitate the collaboration.
- The partnership is **regularly assessed** to celebrate achievements and make any necessary adjustments.

To continue advancing on this front, EURORDIS recommends:

a. formalising patient representation in the ERNs;
b. assessing the impact of patient representatives; and
c. enhancing ERNs' support structures to enable patient partnership.

a. Formalise patient representation in the individual ERNs and in the ERN System

IEB Evaluation Report: *The IEB highlights that "patient representatives need more recognition for their voluntary work as ERN members in the legislation [...] and delineation of patient representatives' roles within the ERNs".*

The report highlights that "*BoMS representatives are in regular contact with ERN coordinators [...]. ERN Coordinators and BoMS representatives also hold periodic meetings with the European Commission".*

Unfortunately, patient organisations and their representatives are not mentioned in this sentence because they are not involved in these periodic meetings to discuss ERN system matters.

Formal recognition and clarity around the role of patient organisations and their representatives would contribute to harmonising the breadth, depth and impact of patient-clinician collaboration across all ERNs.

Additionally, formal recognition would also allow the involvement of patient representatives as equal partners in the transversal cross-ERN working groups, where strategic decisions on the ERN system are made, such as the evaluation of ERNs, the integration of the Networks into national health systems, ethics and legal matters, new calls for affiliated partners and members, etc.

KEY ACTIONS

- The BoMS should adopt in 2025 a **statement to formalise patient representation** in the individual ERNs and in the ERN system.
- The EC should also engage in a dialogue to amend in the mid-term the ERNs legislative acts to cover this aspect.

b. Assess the impact of the contribution of patient representatives

IEB Evaluation Report: *While recognising the efforts of some Networks to assess the involvement of patient representatives, the report stresses that "it is imperative to establish process and outcome metrics to track their real impact. These metrics need to be consistent across networks, so that levels of engagement and collaboration can be routinely assessed. By analysing this data, ERNs should be able to understand the requirements of their collaboration, identify real needs and make changes where necessary."*

To contribute to this effort, EURORDIS has adapted two instruments developed by McMaster University researchers that can be used to measure the impact and implementation of patient partnership.

KEY ACTIONS

- The ERN Evaluation Working Group is encouraged to include the surveys adapted by EURORDIS **in the evaluation toolbox for 2027 to assess the implementation and impact of patient partnership**.
- Individual ERNs are encouraged to use these surveys to monitor on a regular basis their progress towards **implementing patient partnership as part of their monitoring system**.

c. Enhance ERNs' support structure to implement patient partnership

IEB Evaluation Report: *The IEB highlights that ERNs should have "mechanisms for patient engagement, […] and allocation of resources to bolster their efforts". It also suggests "creating concise organisational tools and guidelines that facilitate and streamline the collaboration and ensure the impact of the partnership. These guidelines must specify when and how patients should be involved in various ERN activities".*

Different tools and guidance are already available (e.g. Handbook 13 to involve patient representatives in guideline development, patient journeys methodology and templates, patient partnership framework for the ERNs, EJPRD guide on patient partnerships in rare disease research projects, etc.). EURORDIS has raised awareness and facilitated access to such tools through the Patient Partnership Hub. However, most clinicians involved in the ERNs might still not be aware of the existence of the Hub or its tools, and need training on how to use them. More generally, patient representatives and clinicians need to have or acquire certain skills and competencies to collaborate more effectively, and ERN project managers should have or develop skills to enable this collaboration.

In addition, all ERNs should have an adequate support structure to enable patient partnership. This support structure refers to the capabilities, including the knowledge and skills for shared decision-making and collaboration, the human and financial resources and the organisational processes required to allow health professionals and patient representatives to partner effectively.

KEY ACTION. Individual ERNs should establish a process to **assess each year the effectiveness of their support structure for patient partnership** (capabilities, human and financial resources and organisational processes), identify gaps and discuss specific short/medium and long term actions to address them.

2. DIVERSIFY SOURCES OF FUNDING AND REVIEW THE FUNDING SYSTEM

IEB Evaluation Report: *The IEB recommends undertaking a comprehensive review of the ERNs funding system. To be comprehensive, this review should look at: (i) the sources of funding, (ii) the activities or* *services funded, and (iii) the funding instruments that should ensure business continuity and the smooth operations of the Networks.*

Regarding the sources of funding, only through an adequate mix of funding sources will the ERNs be able to deliver on their ambition. EU budgets, national health budgets and industry funding should all contribute to financing the system.

EU budget to support coordination and core activities: ERNs are, by definition, an EU infrastructure, and as such, the EU budget must continue allocating adequate resources to finance the Coordinating teams and the ERNs core collaborative activities. For example, it is of particular concern to read in the evaluation report that limited funding is the main obstacle, preventing the expansion of thematic groups and the implementation of certain initiatives to increase disease coverage.

National health budgets to support HCPs: As the evaluation report recommends, Member States should allocate financial resources to integrate their health system and the ERNs, addressing the gap between the national health system and each ERN.

Industry funding for research activities: ERNs' potential in the research space has not yet been fully achieved. While ERDERA will certainly help to pave the way in certain aspects, a more forward-thinking vision is required to fulfil this important recommendation issued by the Rare 2030 Foresight Study i.e having "*clear rules that enable European Reference Networks to collaborate with industry across a range of pre-agreed activities, which are clarified and tested through pilots and supported by shared SOPs, to accelerate research and build mutually-agreeable public private partnerships: a central business development/tech transfer office could promote, coordinate and supervise European Reference Networks' interactions and agreements with industrial partners*".

KEY ACTIONS

- The **2028-2035 EU MFF** should maintain an adequate level of funding to cover the Networks' coordination expenses as well as training and education activities, the development or revision of clinical practice guidelines and other clinical decision-support tools and the networking activities. This budget should have a direct link and be proportionate to the size and scale of ambition and activities of each ERN, through a 'cost and volume' model, combining a fixed payment of the same amount for all Networks regardless of size (core structural costs, including administrative and management support, translation, etc.), complemented with an additional payment proportionately based on the size of the Networks (networking activities and logistics). Additional non-competitive funding should be allocated to ERNs registries, to enable large scale data collection and analysis as well as their full participation in the EHDS.
- **Member States should increase their financial commitment** to the ERN system, through financial and in-kind support to all the Expert Centres involved in the Networks.

- If **industry collaboration in ERNs' research** activities is expected, the ERNs BoMS, Commission and ERN Coordinators should **co-develop** with the patient representatives involved in the Networks a single legal framework and model to operationalise such a collaboration under clear and transparent rules to ensure the prerogative of each ERN to decide on the use of funding.

3. INTENSIFY THE FOCUS ON INTEGRATION

a. ERNs to support implementation of CPGs and CDSTs and monitor adherence

IEB Evaluation Report: *The report recommends ERNs to "refine their oversight strategies to monitor the implementation of the guidelines, pathways, and protocols as well as the adherence to them among their members".*

Apart from refining these strategies, ERNs should also intensify their efforts to support the implementation of CPGs and CDSTs and best practice at the local level. A central support implementation team composed by local leads (clinicians from ERN HCPs in different countries) and ERN Coordination staff, such as the one established by ERNICA ERN, could perform this supporting function. This approach to bridge the gap with the national health systems would contribute to improving outcomes of care and show the commitment of the ERNs with the integration agenda.

KEY ACTION. All ERNs should establish an implementation support team in 2025 to support implementation of CPGs and CDSTs at local level and monitor adherence.

b. National health and regional health authorities to implement their integration strategy

IEB Evaluation Report: *According to the evaluation report, more than 2 million patients were diagnosed and treated by HCPs during the evaluation period (5 years). However, this represents only a small percentage of the 30 million people who live with a rare disease in Europe.*

While it is great that more than 2 million people have been diagnosed or treated directly in ERNs Expert Centres, the system must also benefit people who will be diagnosed and treated by healthcare providers that do not belong to an ERN. This is why their integration into national health systems is vital for the Networks to deliver on their ambition and for their sustainability.

The ERN system should aim at improving equity in health outcomes and access to evidence-based healthcare by building on or enhancing national health system capacities to diagnose and treat rare and complex conditions. Health authorities may pursue this through a combination of different approaches and actions such as designating affiliated partners, implementing new or adapting existing national and cross-border care pathways, establishing national reference networks or other mechanisms to interface with the ERNs, revitalising rare disease plans or strategies, etc.

While representatives from the Member States involved in JARDIN will work to generate some of these tools and recommendations, the success of this process largely depends on the commitment in all EU countries beyond JARDIN and the capacity of decision-makers and other stakeholders to utilise and integrate those tools and recommendations, establish a clear vision and strategy, mobilise resources and drive reforms where needed.

KEY ACTION. By 2027, all EU countries should have started implementing their own **ERNs integration strategy (governance mechanisms, infrastructures and action plan),** to effectively use the ERNs to improve health outcomes of their population and increase local access to evidence-based healthcare for rare and complex conditions.

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4. MEASURE OUTCOMES AND CLINICAL PERFORMANCE

a. Measure and monitor Patient Experience with care

IEB Evaluation Report: *The report recommends that HCPs should measure patient experience with care and suggests doing so using a methodology that is consistent across Networks. One such method that can be used to measure patient experience is via patient reported experience measures (PREMs). PREMs can be insightful assessment tools of quality care for people living with a rare disease (PLWRD), ensuring healthcare delivery responds to their needs and preferences.*

As the IEB explains, "patient experience, including the systematic collection and regular analysis of this feedback by HCPs, is an important component of patient-centred care. This practice gives patients a clear voice and provides valuable insights for making improvements from the patients' point of view. It also addresses issues that the team may not be aware of. ERNs should encourage HCPs to regularly use a standardised tool and analyse the results to identify opportunities to improve the patient experience of their members. It is recommended that a validated methodology be developed to collect and analyse the experiences of patients receiving care in specialised units. Ideally, this methodology should be consistent across networks".

The Irish Rare Disease Research Catalyst Consortium (RDCat) is developing a generic PREM for patients and carers living with a rare condition, leveraging on the work done by EURORDIS on this area since 2020. It is expected that the Consortium will deliver a validated PREM in English in 2026. However, the collaboration of the ERNs with RDCat would help to accelerate the development of the PREM and financial support from the Commission to translate the resulting PREM in 24 languages would allow it to be used by HCPs across the EU in 2026.

KEY ACTIONS

- **ERNs are encouraged to engage in 2025 with RDCat** to support the validation of the generic PREM in English.
- The 2027 Evaluation should **include a measurable element for HCPs/institutions** that refers to having a team in the hospital dedicated to drive quality improvement using feedback on the experience of patients with care.

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b. Measure and monitor outcomes of care and clinical performance

IEB Evaluation Report. *The report recommends ERNs develop a comprehensive set of measures or indicators to monitor clinical processes, performance, and outcomes of care. With this information regularly collected from their Expert Centres, ERNs would be encouraged to analyse these indicators on a regular basis to stimulate collective reflection for learning and improvement.*

The ERN evaluation and monitoring system should include measures on clinical performance and outcomes of care. The current lack of such measures is a lost opportunity, as collecting such information from the Expert Centres allows them to review and compare their diagnostic, therapeutic or surgical performance, as well as patient outcomes, against the ERN average. This provides Expert Centres with valuable actionable information to adjust and improve their own clinical practice that will drive increases in the average and stimulate a process of continuous clinical innovation and improvement in all Centres.

KEY ACTION. In 2025, all ERNs should define and start **collecting from HCPs disease-specific KPIs on clinical performance and outcomes of care as part of their monitoring system**. Collecting outcome measures in a consistent and interoperable manner is an essential foundation to build the Networks' ability to foster innovation of clinical practice and learning among the Expert Centres under each ERN.

5. QUALITY IMPROVEMENT SYSTEM THAT CONNECTS ASSESSMENT, MONITORING & EVALUATION

IEB Evaluation Report. *The* report recommends "*to establish an integrated quality management system to help ERNs identify trends, targets and opportunities for improvement using a standardised methodology. This system should effectively link the analysis of indicators to the objectives of the network, taking into account the objectives and KPIs set out in the technical reports of the grant*".

The call for tender CHAFEA/2019/HEALTH/18 Service contract for the provision of an integrated assessment, monitoring, evaluation and quality improvement system (AMEQUIS) for the European Reference Networks included specifically the development of an Integrated Continuous Quality Improvement system and toolbox. This system should integrate the monitoring system of the ERNs with the assessment and evaluation processes in a continuous quality improvement cycle of actions.

This Quality improvement system was never published, however ERNICA has developed a quality cycle for continuous improvement, and ERKNet uses data collected from the Expert Centres on key performance indicators to identify areas for improvement and take action. It is within the power of individual ERNs to develop such a system, should they choose to do so, as a tool to guide their actions and work plans.

KEY ACTION. The ERNs should **adopt in 2025 a Quality Improvement System** following a Plan-Do-Check-Act approach for continuous quality improvement.

3. SUMMARY TABLE KEY ACTIONS

AREA OF IMPROVEMENT	KEY ACTIONS
Formalise and implement patient partnership in the ERNs	1. The ERNs BoMS should adopt in 2025 a statement to formalise patient representation in the individual ERNs and in the ERN system. The EC should also engage in a dialogue to amend in the mid-term the ERNs legislative acts to cover this aspect. 2. The ERN Evaluation Working Group is encouraged to include the surveys adapted by EURORDIS in the evaluation toolbox for 2027 to assess the implementation and impact of patient partnership. 3. Individual ERNs are encouraged to use these surveys to monitor on a regular basis their progress towards implementing patient partnership as part of their monitoring system. 4. Individual ERNs should establish a process to assess each year the effectiveness of their support structure for patient partnership (capabilities, human and financial resources and organisational processes), identify gaps and discuss specific short/medium and long term actions to address these gaps.
Diversify sources of funding and review the funding system	1. The 2028-2035 EU MFF should maintain an adequate level of funding (at least €1 million per year, per ERN) to sustain the ERNs' operations. This budget should have a direct link and be proportionate to the size and scale of ambition and activities of each ERN, through a 'cost and volume' model, combining a fixed payment of the same amount for all Networks regardless of size (core structural costs, including administrative and management support, translation, etc.), complemented with an additional payment proportionately based on the size of the Networks (networking activities and logistics). 2. Member States should increase their financial commitment to the ERN system, through financial and in-kind support to all the Expert Centres involved in the Networks.

AREA OF IMPROVEMENT	KEY ACTIONS
	3. If industry collaboration in ERNs' research activities is expected, the ERNs BoMS, Commission and ERN Coordinators should co-develop with the patient representatives involved in the Networks a single legal framework and model to operationalise such a collaboration under clear and transparent rules to ensure the prerogative of each ERN to decide on the use of funding.
Intensify the focus on integration	1. All ERNs should establish an implementation support team in 2025 to support implementation of CPGs and CDSTs at local level and monitor adherence. 2. By 2027, all EU countries should have started implementing their own ERNs integration strategy (governance mechanisms, infrastructures and action plan), to effectively use the ERNs to improve health outcomes of their population and increase local access to evidence-based healthcare for rare and complex conditions.
Measuring outcomes of care and clinical performance, instead of outputs	1. ERNs are encouraged to engage in 2025 with RDCat to support the validation of the generic PREM in English. 2. The 2027 Evaluation should include a measurable element for HCPs/institutions that refers to having a team in the hospital dedicated to drive quality improvement using feedback on the experience of patients with care.
Developing a quality improvement system	1. The ERNs should adopt in 2025 a Quality Improvement System following a Plan-Do-Check-Act approach for continuous quality improvement.