Press release

Support for Commission's 'most successful innovation' of expert rare disease networks must continue

***3 October, Brussels* – EURORDIS-Rare Diseases Europe and representatives from 24 European Reference Networks (ERNs) have written to the leaders of the European Commission, European Parliament and EU Member States to insist on the renewal of key support for the Networks.**

ERNs are a vital component of Europe's healthcare landscape, connecting clinical experts from different countries to collaborate on the diagnosis, treatment, and management of rare diseases. These networks have made huge strides in addressing the unique challenges posed by rare and complex conditions.

Thus far, the European Commission, including the Health Commissioner Stella Kyriakides, and the EU institutions have shown commendable leadership in their steadfast support for the ERNs, recognising the strategic opportunity of enhanced EU cooperation in this area. In a recent response to a letter from the European Parliament, the President of the European Commission, Ursula von der Leyen herself, described the ERNs as the "the most successful innovation" in the Commission's efforts to make sure all rare disease patients "have access to the best possible knowledge, diagnosis, and treatment".

Due to the often small patient populations and complexity of these conditions, people living with a rare or complex disease frequently face immense challenges in accessing desperately needed clinical expertise within their nations. The consequences of this reality include poor access to diagnoses, treatments, or other appropriate healthcare or social actions.

To respond to these challenges, the ERNs were first launched in 2017 with the goal of connecting healthcare professionals from different countries, enabling them to pool their resources, share patient data, and develop best practices. This approach helps ensure that patients with a rare or complex condition receive high-quality care and have access to the most up-to-date medical knowledge and treatments, regardless of where they live in Europe.

Discussing the open letter from EURORDIS and the ERNs, **Ines Hernando**, ERN and Healthcare Director at EURORDIS, said:

> "For the tens of millions of people across Europe with rare and complex diseases, who have long faced the challenges of misdiagnosis, delayed treatment, and isolation, the European Reference Networks have been a bright beacon of hope.
>
> "ERNs have shattered some of the barriers that once separated patients from expertise, providing a platform where healthcare professionals, researchers, and patient organisations are united in their mission to conquer the healthcare challenges that are inherent to rare and complex diseases.

"Yet, these networks are not just about medical collaboration, as they are also about rewriting the narratives of people's lives. They are about giving every person with a rare or complex condition in Europe the equitable opportunity to access timely, high-quality specialised healthcare. While the successes of ERNs speak for themselves, we urge the EU institutions to continue their unwavering support for ERNs.

"If the ERNs are now adequately supported to become fully integrated into national health systems, we will see a modernisation of healthcare pathways for rare and complex conditions, which make a profound difference in people's lives."

Also speaking about the letter, co-signatory **Dr. Holm Graessner**, Coordinator of the European Reference Network for Rare Neurological Diseases (*ERN-RND*), said:

"In the face of the COVID-19 pandemic, ERNs swiftly harnessed their collective wisdom, offering crucial guidance to protect the most vulnerable among us. They have facilitated cross-border expert panels to discuss complex cases, developed valuable resources for clinicians and people living with rare and complex conditions, and training programmes to counter the effects of workforce shortages in our field.

"The Commission President herself has rightly celebrated the ERNs as the most successful innovation among the EU's rare disease policies. However, our ERN community and rare disease patients must emphasise to EU policymakers that these networks have not yet realised their full potential.

"To achieve their maximum impact, ERNs undeniably need long-term financial stability and sustained resource commitments, a formal role in EU cross-border decision-making, and increased support for recruiting and retaining participants across patient organisations, healthcare providers, and clinicians."

In total, there are currently 24 ERNs, which are each dedicated to a specific category of rare diseases, such as bone disorders (*ERN BOND*), childhood cancers (*ERN PaedCan*) and hereditary metabolic disorders (*MetabERN*). Today, the ERNs collectively bring together more than 1,600 highly specialised healthcare units from 27 EU countries and Norway.

In the open letter, the signatories highlight several **key achievements** of ERNs. These include the:

- **Pooling of expertise during COVID-19:** ERNs were able to unite their collective expertise to provide expert opinions on COVID-19 vaccination priorities and contraindications for adult patients with rare or complex conditions, demonstrating their agility and responsiveness in times of crisis.
- **Facilitation of cross-border expert panels:** Over the past six years, ERNs' experts have participated in virtual cross-border panels to discuss more than 3,450 cases, allowing specialised knowledge to transcend national boundaries for the benefit of patients.
- **Development of Clinical Practice Guidelines (CPGs):** ERNs have developed, appraised, and endorsed over 400 clinical practice guidelines, ensuring that the latest standards of care are available for adoption by hospitals and treating clinicians. .
- **Creation of Patient Registries and Research:** ERNs have established patient registries, with over 50,000 rare disease patients currently enrolled, and have taken steps to measure patient experience with healthcare. These initiatives are expected to drive improvements in care and research.

The letter also outlines a series of **recommendations** for the future, which include:

- **Securing long-term financial stability,** through Ensuring that ERNs have the necessary resources to continue their critical work.
- **Covering geographic gaps** through the **designation of 'Affiliated Partners'** , where needed.
- **Expanding the disease coverage** of the ERNs, while ensuring good quality assurance in this process as the Networks expand their scope.
- **Enhancing cross-ERN collaboration,** through addressing multi-systemic rare conditions and areas of common interest more effectively.
- **Establishing treatment eligibility and molecular therapeutic panels,** which enable ERNs to play a formal role in EU cross-border healthcare decision-making.
- **Committing to fully integrating the Networks into national health systems,** through allocating adequate human and financial resources to implement and use the recommendations and resources that will stem from the future Joint Action on Integration of ERNs.
- **Monitoring patients' s experiences and health outcomes,** by collecting and monitoring experienced-based and outcome measures to improve people's experience with specialised healthcare services and to assess the impact of ERNs on patient outcomes.
- **Supporting Volunteer Contributions, through recognising** the vital role of patient organisations, representatives, healthcare providers, and clinicians in ERNs and addressing challenges in recruitment and retention.

Ultimately, and as expressed in this letter, the rare disease community calls on EU institutions and Member States to seize this moment to further consolidate ERNs, transforming them into a flagship of the European Health Union. The commitment of EURORDIS, the ERNs, and the wider rare disease community to ensuring equal access to timely and specialised healthcare for individuals living with rare or complex conditions remains unwavering.

Read the letter from EURORDIS & representatives from the European Reference Networks to EU leaders.

About EURORDIS-Rare Diseases Europe

EURORDIS-Rare Diseases Europe is a unique, non-profit alliance of over 1,000 rare disease organisations from 74 countries that work together to improve the lives of the 30 million people living with a rare disease in Europe. By connecting people, families and rare disease groups, as well as by bringing together all stakeholders and mobilising the rare disease community, EURORDIS strengthens the patient voice and shapes research, policies and services.

Contact

Julien Poulain
Communications Manager
EURORDIS-Rare Diseases Europe
Julien.poulain@eurordis.org
+33 6 42 98 14 32