

The rare disease community comes together in calling on the EU institutions and our national governments to stand by the European Reference Networks.

Dear Mrs von der Leyen,
President of the European Commission,

Dear Mrs Metsola,
President of the European Parliament,

Dear Heads of Governments of EU Member States,

We, the undersigned, representing the European Reference Networks (ERNs) and people living with a rare disease across Europe, **call on the EU institutions and EU Member States' governments to stand by the European Reference Networks** and to uphold their commitment to enable long-lasting impact in people's lives and, fundamentally, give all people living with a rare or complex condition in Europe the same opportunities to access timely and adequate specialised healthcare.

The European added value to addressing the needs of people living with a rare disease is exceptionally high, due to the rarity of patients, experts, knowledge, data and resources. The European Union and its Member States have fostered tremendous progress in this area, and the establishment and support of the Networks' endeavours prove that Europe has, and continues to be, a global leader in highly specialised healthcare innovation. We **applaud the steadfast leadership from the European Commission and the Health Commissioner, and the political support from the EU institutions** that have recognised the strategic opportunity of enhanced EU cooperation to address the healthcare needs of people with rare and complex conditions.

The launch of the Networks in 2017 established a formal permanent infrastructure for knowledge creation and sharing, structured collaboration, partnership between experts and people living with a rare disease and virtual expert advice across EU borders. The current EU financial framework allocated significant funding to deploy the ERNs and further strengthen their capacities as a permanent infrastructure for cross-border healthcare collaboration. As highlighted by the President of the European Commission in a recent response to a letter from the European Parliament, "at the very core of our efforts is the determination to ensure that all rare disease patients, including children, have access to the best possible knowledge, diagnosis, and treatment. **The European Reference Networks proved to be the most successful innovation in the area and key to achieving this goal.**"

As patient representatives and clinicians, we have seen the positive impact that the Networks have had for the rare disease community.

As a response to tackle the recent COVID-19 pandemic, as early as in March 2020, the 24 ERNs were able to pool their collective expertise and knowledge, and publish their expert opinion on the priorities and contraindications of COVID-19 vaccination for adult patients with a rare or complex condition falling under the scope of each Network.

Over the last six years, the Networks' experts have **discussed and provided virtual EU cross-border consultation on diagnosis, treatment and disease management for more than 3,450 cases**. This has allowed expertise, rather than people, to travel across borders for the delivery of high quality care. During this time, the Networks have also developed, as well as appraised and endorsed, **more than 400 clinical practice guidelines** and have produced

valuable resources in all EU languages for people living with a rare and complex condition. **Structured training and education programmes for young clinicians and healthcare professionals** have been set up to counter the decreasing numbers of rare disease experts.

Moreover, all ERNs have established **patient registries** with the ambition of improving care and fostering research. More than 50,000 patients with rare diseases are currently followed in these registries, with an expected five-fold increase in the number of patients over the next three years. A repository of **patient reported outcome measures** relevant to rare and complex conditions is now publicly available and will facilitate the use of these measures in clinical research and to improve healthcare. The ERNs have also taken the first steps to measure **patients' experience with healthcare** using a shared instrument across countries and diseases.

The Networks have the potential to harness EU solidarity for enhancing health equality across our countries and expanding our health systems' capabilities to diagnose, treat and manage rare and complex conditions. Nevertheless, ERNs have not yet fully delivered on their potential and we know there is still a long way to go to achieve health equality and equity across the European Union for the vulnerable population they serve.

The next EU political cycle must succeed in fully consolidating the Networks and their seamless integration into cross-border and national healthcare systems. The time is ripe to move the ERNs from good to great and showcase this model of solidarity and cooperation as world-leading in highly specialised healthcare. We are therefore calling on EU institutions and Member States to:

- **Secure long-term financial stability** for the Networks.
- **Designate Affiliated Partners**, where needed, to ensure that all EU health systems collaborate with all 24 ERNs and expand the **disease coverage** to cover new conditions through a mechanism that ensures a rigorous and transparent process to establish specific criteria and the assessment of new applicants and existing full members that wish to validate their expertise to treat and manage the new diseases.
- **Support greater cross-ERN collaboration** both to tackle the challenges of multisystemic rare and complex conditions and address areas of common interest more effectively.
- **Establish treatment eligibility and molecular therapeutic panels** that would issue binding advice on access to cross-border highly specialised healthcare and enable ERNs to play a **formal role in the EU cross-border healthcare decision-making** process.
- **Fully integrate ERNs into national health system services** and infrastructures, implementing the future recommendations of the Joint Action on Integration (JARDIN) through a whole-systems approach to planning, delivery and evaluation of highly specialised health services.
- **Urgently define and systematically collect and monitor health and experienced-based outcome measures** that assess Networks' progress towards improving health outcomes and the lives of people living with a rare or complex condition.

- **Better support and recognition of the contribution of volunteer patient organisations, patient representatives, healthcare providers and clinicians in the Networks** to guarantee continuous and active participation in their collaborative activities. This is needed as the recruitment and retention of clinicians and patient representatives who are voluntarily contributing to the development of the ERNs is an emerging challenge.

We, the undersigned, stand together, united in our belief in the collective capacity and capability of the Networks. We are unwavering in our commitment to spearhead the consolidation of well-integrated ERNs. We believe that the above actions are crucial to consolidate the ERNs and turn them into a flagship of the upcoming European Health Union.

As a new EU political cycle approaches, **we call on the EU institutions and EU Member States' governments to stand by the European Reference Networks** and to uphold their commitment to enable long-lasting impact in people's lives and, fundamentally, give all people living with a rare or complex condition in Europe the same opportunities to access timely and adequate specialised healthcare.

Yours sincerely,

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