

# How the European Commission works on Rare Diseases and the European Reference Networks (ERNs)

February 2025



## Did you know?



The work on **Rare Diseases** involves many Commission Directorate-Generals (DGs) and services, which **coordinate together the work and expertise to advance the EU's activities on rare diseases in a multi-disciplinary and collaborative manner.**

## Framing the EU's work on rare diseases



Health policy and the organisation of delivery of health services and medical care is a national competence. However, the Commission complements national policies, **encouraging the exchange of best practices** and **cooperation** across **Member States**.



## The Commission acts under the framework of the following:

- [Commission Communication on Rare Diseases](#)
- [Council Recommendation in the field of rare diseases](#)
- [Cross-border healthcare Directive](#).

This sets out the parameters of work across the EU on rare diseases from the public health perspective. Regarding [research](#), the EU has long prioritised support for rare diseases through successive research and innovation framework programmes, with the strong involvement of the ERNs in several [projects](#) and clinical trials.



## Orphan medicines and medical devices

The EU acts to **improve access to safe and more effective orphan medicines** and medical devices. For orphan medicines, the Commission has revised the orphan legislation and proposed changes to address all the challenges identified in the last 20 years.

The changes aim to promote the development of orphan medicines for underserved rare diseases via targeted incentives, enhanced regulatory support and improved equal access to orphan medicines.

In 2024, [Guidance](#) on the clinical evaluation of orphan medical devices was provided to assist manufacturers in meeting the regulatory requirements in the EU Medical Devices Regulation. Based on that Guidance, the European Medicines Agency launched a pilot programme to support the development and assessment of orphan medical devices through the involvement of expert panels.



## Data availability and data sharing

The forthcoming [European Health Data Space](#) will **improve** the continuity of care for patients at the national and European level by **enabling secure access and sharing of electronic health records across the EU**. The European Health Data Space will support the secondary use of data by making more data on rare diseases available for research and innovation. The improved access to data is expected to accelerate the development of new treatments and medicines for rare disease patients.



## European Reference Networks (ERNs)

The Commission is **reinforcing** the work of the [24 European Reference Networks](#) (ERNs), which are cross-border networks that bring together European centres of expertise and reference to tackle rare, low prevalence and complex diseases and conditions requiring highly specialised healthcare. The **ERNs represent a key element** of the European Health Union in rare diseases, working on network coordination, patients' registries, training, clinical practice guidelines and communication to raise awareness on rare diseases and ERNs activities.

# How is the governance of the European Reference Network organised?



## EU engagement with Member States

The [Board of Member States](#) is a group of national representatives overseeing the European Reference Networks. Their responsibilities include **approving** or **terminating** networks and membership applications. Twice a year, the Commission organises meetings with the Board of Member States to steer the ERNs' work and, when necessary, find consensus on decision making elements. Additional meetings may be organised on specific topics relevant to the ERNs.



## EU engagement with the European Reference Networks

The Commission also works closely with the Networks with **meetings organised twice a year** with the [ERN Coordinators Group](#). This group brings together each ERN's coordinator and addresses common points across all Networks on technical and organisational aspects. Given their expertise, they are also invited to advise the Member States and the Commission.

## Working together with



## Patients' organisations

Keeping **the voices of patients and families at the centre of the EU work in rare diseases is also essential**. The Commission therefore collaborates with patient organisations, notably [EURORDIS](#), the non-profit alliance representing over 1,000 rare disease patient organisations. For instance, patient organisations play a vital role within the work of the European Reference Networks and EURORDIS is an important partner in many EU-funded projects such as [ERDERA](#) and [Screen4Care](#).



## Orphanet

The Commission also **supports Orphanet**, an initiative that manages a digital portal for gathering and improving knowledge on rare diseases to **improve diagnosis, care and treatment** of patients with rare diseases. In addition, Orphanet maintains the rare disease nomenclature known as [ORPHAcode](#) which allows the harmonised codification of rare diseases worldwide.



## Supporting Ukraine on tackling rare diseases

The Commission stands by Ukraine, including in the field of rare diseases. A framework was put in place for Ukrainian healthcare providers to seek advice from members of the European Reference Networks on Ukrainian rare or complex disease patients.

Moreover, the ERNs undertake collaboration activities, capacity building and best practice sharing for competent Ukrainian authorities and healthcare units.

