Orphan Drug Development Guidebook

Building Block E125

This document defines the content of the Building Block created for each identified tool, incentives, initiative or practice introduced by public bodies or used by developers to expedite drug development in Rare Diseases (RDs).

ITEM	DESCRIPTION
Building Block (BB) Title	European Reference Networks (ERN)
References	https://webgate.ec.europa.eu/ern/
Description	ERNs are virtual, voluntary cross-border networks, bringing together highly specialised healthcare providers across Europe to help diagnose and treat patients suffering from rare or low prevalence complex diseases that require highly specialised healthcare and a concentration of knowledge and resources. The participation of the ERNs in the research effort provides patients with access to cutting-edge treatments and benefit the population and the health services as a whole by leading to the development of more effective, high quality, cost-efficient treatments and healthcare delivery models. Integration and Complementation with existing Clinical Research Organizations in the USA and in EU (i.e. ERNs) will enhance the quality of research and development, clinical trials by partnering with and providing the stakeholders with forward-thinking, cost effective methods to develop new drugs. ERNs represent an unicum in the clinical and research worldwide collaborative panorama, indeed 24 ERNs span the over 6000 rare diseases by involving 300 EU HCPs and over 1000 specialized units in 25 EU countries. ERNs are derived from the application of Art. 12 of the EU Cross Border Directive EU24/2011. EUCERD (European Union Committee of Experts on Rare Diseases) did categorize rare diseases in 21 fields. For each of the identified fields, the EC (European Commission)

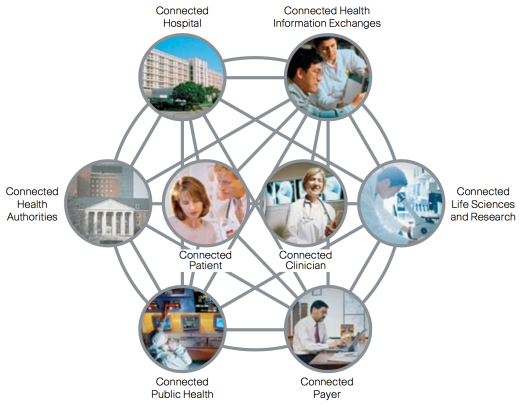
was calling to generate one ERN, formed by centers of excellence HCPs (Health Care Providers), participating as Full Member Providers, endorsed by the local Ministry of Health (or competent agency) and complying with ERNs´ enrolment criteria.

The 24 Coordinators are forming the ERN-Coordinators Group (ERN-CG) which is a formal body of the EC aimed at the optimization of the implementation of the program and as expert support to the EC.

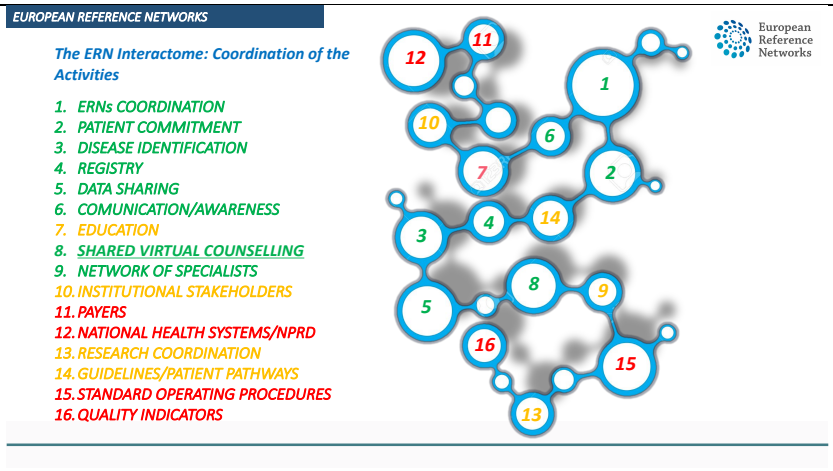
ERNs are patient centered, multidisciplinary organizations conducting mainly clinical, research and health policy activities. The HCPs have ongoing longstanding collaboration with the major research groups and infrastructures (i.e. BBMRI; Elixir, ECRIN, etc.) for the development of new diagnostic methods, therapies and innovative technologies.

Aim of the ERN:

Structure of the ERN:



Interactome diagram of Activities of the ERNs. Color code: Green: achieved; Yellow: in the process; Red: to be still pending;

	
Category	Developmental Resources Building Block
Geographical scope	European Union
Availability	Applicants developing medicines for rare diseases.
Scope of use	– To implement the know-how for innovative drugs development – To implement and complement expertise – To collaborate with Patients Organizations for the development of new scientific and clinical methodologies – To foster basic and applied research – To optimize use of scientific and technical infrastructures – To optimize human and economical resources – To protect the future of Rare Diseases through ensuring new generations of medical professionals – …
Stakeholders	• Healthcare professionals, • Pharmaceutical industries, • Patient organizations, • Policy makers and payers, • EC/NIH/FDA/EMA´s representatives for Research

Enablers/ Requirements	Enablers: ERNs representatives, Patients Organizations and Pharmaceutical Industries, Health Policy Makers and representatives of the EC, Payers. Requirements: • Inform (e.g. regarding the vision/mission and expected impact of the networks); • Consult (written – e.g. surveys); • Consult and involve (direct interactions – e.g. stakeholder meetings, workshops, stakeholder conferences); Cooperate / participate (direct interactions - e.g. creation of topic-specific working groups)
Output	Generation of recommendation regarding the unmet need of patients about research, generation of collaborative research projects at basic and applied levels.
Best time to apply and time window	The tool has its best use as early as possible.
Expert tips	This can include DOs and DON'Ts and strategic considerations. PROs: – Optimization of resources, optimal critical mass, sustainable programs for research, – Optimization of infrastructure use, – Focused and non-redundant research projects – Better programming of research investment CONs: – Lack of Legal Entity – Lack of integration with the NHS of the Member States – Scarcity of Funds