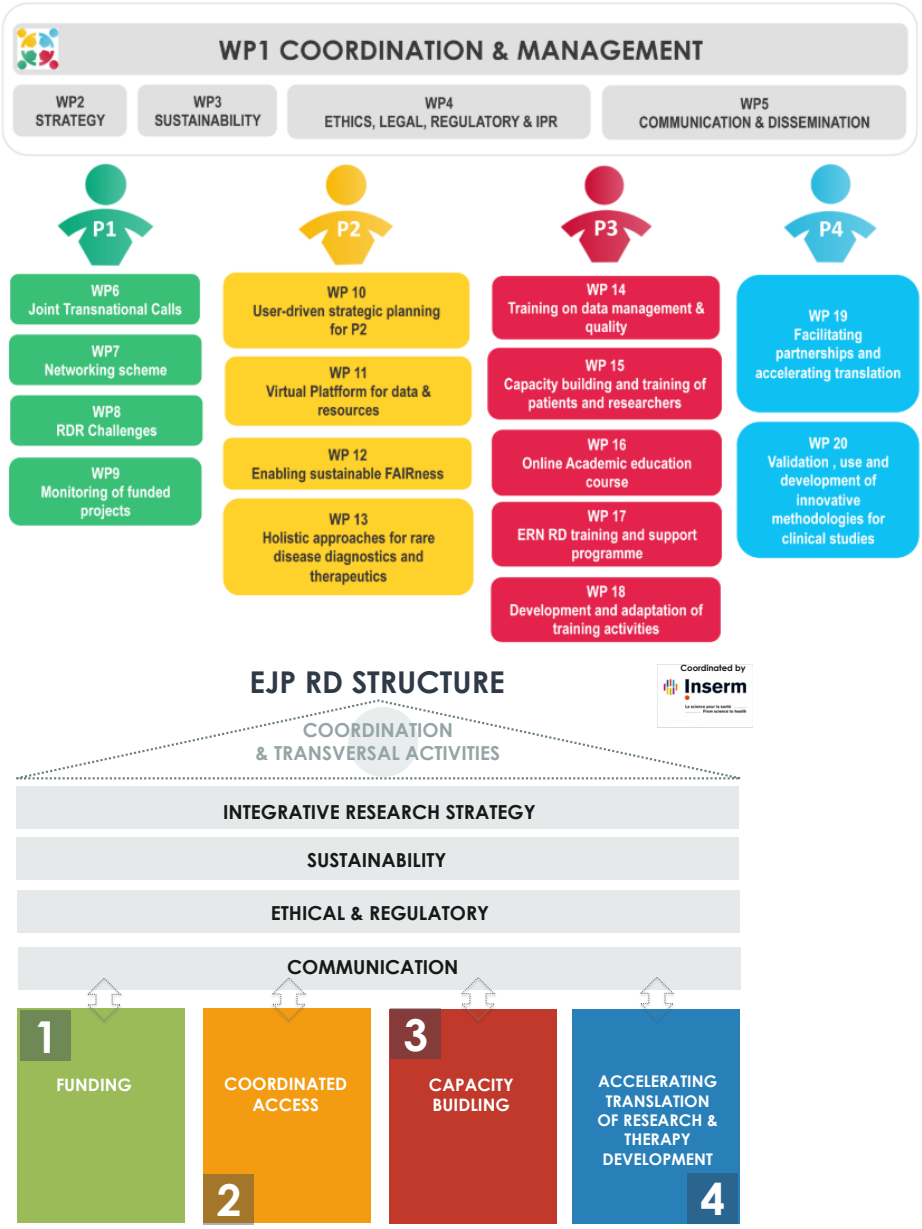
Orphan Drug Development Guidebook

Building Block E124

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 Joint Program on Rare Diseases
References	https://www.ejprarediseases.org/ H2020-SC1-BHC-2018-2020, Topic: SC1-BHC-04-2018 Type of action: COFUND-EJP, Proposal number: SEP-210488440
Description	The European Joint Programme Cofund (EJP-RD) is an instrument allowing high level strategic organization and performance of research activities in an organized and transversal manner. Participation of Programme Owners (ministries) and Programme Managers (Research Funding and Research Performing organizations) accompanied by other relevant stakeholders (e.g. patients' organizations, ERNs, regulatory bodies and private sector) will ensure the necessary level of integration and unique strategy to efficiently tackle societal challenges. The ambition of the EJP-RD is to improve alignment of national/regional activities and policies in rare disease, improve lives of rare disease patients by providing new and optimised treatment options and diagnostic tools for these diseases, decrease fragmentation of rare diseases expertise and research resources, increase the EU's capacity to innovate in the field of rare diseases, improve healthcare systems' capacity to take up research results, reinforce the EU's role as a global leader for rare diseases, follow the policies and contribute to the objectives of the International Rare Diseases Research Consortium (IRDiRC), contribution to the European Open Science Cloud. The project is designed in 5 Pillars : Pillar 0: Coordination, Transversal Activities & Communication Pillar 1: Fundings and Calls Pillar 2: Coordinated Access to Data and Services

	Pillar 3: Training and Empowerment Pillar 4: Innovation and Clinical Trials Support 
Category	Development Opportunity Building Block
Geographical scope	30 institutions (including all 24 ERNs) from 35 countries: 26 EU Member States (Austria, Belgium, Bulgaria, Czech Republic, Denmark, Estonia, Finland, France, Germany, Greece, Hungary, Croatia, Ireland, Italy, Netherlands, Latvia, Lithuania, Luxembourg, Malta, Poland, Portugal, Romania, Spain, Sweden, Slovakia, Slovenia)

	7 associated (Armenia, Georgia, Israel, Norway, Serbia, Switzerland, Türkiye) Uk & Canada
Availability	European Research Infrastructures European Reference Networks (ERNs) Patient & Non-profit organizations Research Institutes Research Funding Organisations Universities working on rare disease and low prevalence disease
Scope of use	• Improve the integration, the efficacy, the production and the social impact of research on RD through the development, demonstration and promotion of Europe/world-wide sharing of research and clinical data, materials, processes, knowledge and know-how • Implement and further develop an efficient model of financial support for all types of research on RD (fundamental, clinical, epidemiological, social, economic, health service) coupled with accelerated exploitation of research results for benefit of patients.
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, Policy Makers and 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	Expected impacts : • Improved lives of RD patients by providing new and optimised treatment options and diagnostic tools • Decreased fragmentation of RD expertise and research resources • Increased EU's capacity to innovate in the field of RD

	• Improved healthcare systems' capacity to take up research result
Best time to apply and time window	The tool has its best use as early as possible and throughout the development process.
Expert tips	PROs: – Optimization of resources, sustainable programs for research, optimization of infrastructure use, focused and non-redundant research projects. Better programming of research investments. CONs: – Optimized coordination with ERNs