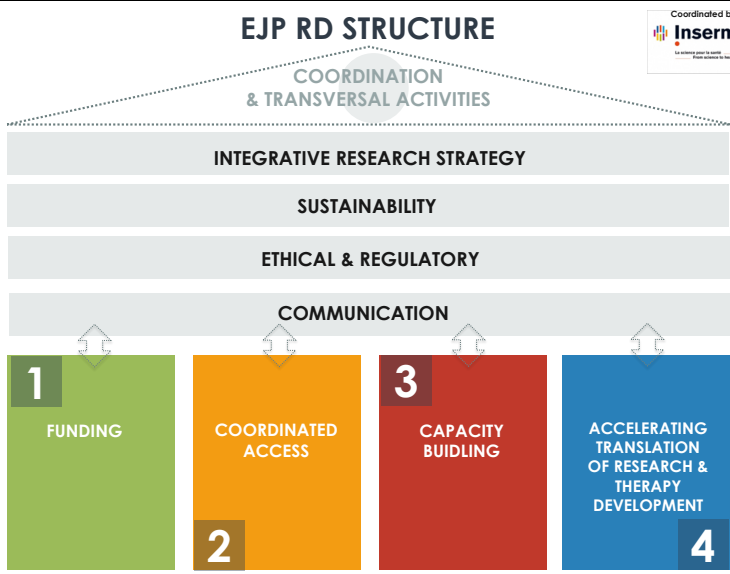


Orphan Drug Development Guidebook

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	<p>The European Joint Programme Cofund 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 European Joint Programme for RD is to establish an urgently needed comprehensive strategy covering research, tools and clinics leading to optimization and exploitation of results, faster drug discovery at reduced costs, improved patients’ care as well as giving Europe a leading role in the field of RD in the coming years.</p> <p>The project is designed in 4 Pillars and the duration of 5 years (2019-2024):</p>

	<p style="text-align: center;">EJP RD STRUCTURE</p> 
Category	Development Opportunity Building Block
Geographical scope	<p>European Union</p> <p>[85 partners and linked third parties from 23 European (AT, BE, CZ, DE, ES, EE, FI, FR, HU, IE, IT, NL, LT, LV, LU, PL, PT, RO, SE, SK, SV, UK), 4 associated (AM, CH, IL, TK) and 1 non-EU (CA) countries + Japan and Australia as observers]</p>
Availability	Only for Public/ no-profit organizations working on Rare Diseases and low prevalence disease.
Scope of use	<ul style="list-style-type: none"> • Create a research and innovation pipeline "from bench to bedside" ensuring rapid translation of research results into clinical applications and uptake in healthcare for the benefit of patients • Follow the policies and contribute to the objectives of the International Rare Diseases Research Consortium (IRDIRC) • Improve integration, efficacy, production and social impact of research on rare diseases through the development, demonstration and promotion of sharing of research and clinical data, materials, processes, knowledge and know-how, and to implement and further develop an efficient model of financial support for research on rare diseases including basic, clinical, epidemiological, social, economic, and health service research
Stakeholders	<ul style="list-style-type: none"> • Healthcare professionals,

	<ul style="list-style-type: none"> • Pharmaceutical industries, • Patient organizations, • Policy makers and payers, • EC/NIH/FDA/EMA's representatives for Research
Enablers / Requirements	<p>Enablers: ERNs representatives, Patients Organizations and Pharmaceutical Industries, Policy Makers and Payers.</p> <p>Requirements:</p> <ul style="list-style-type: none"> • 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	<p>Expected impacts :</p> <ul style="list-style-type: none"> • 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	<p>The tool has its best use as early as possible and throughout the development process.</p>
Expert tips	<p>PROs:</p> <ul style="list-style-type: none"> – Optimization of resources, sustainable programs for research, optimization of infrastructure use, focused and non-redundant research projects. Better programming of research investments. <p>CONs:</p> <ul style="list-style-type: none"> – Optimized coordination with ERNs