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).

<table>
<thead>
<tr>
<th>ITEM</th>
<th>DESCRIPTION</th>
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<tbody>
<tr>
<td>Building Block (BB) Title</td>
<td>European Joint Program on Rare Diseases</td>
</tr>
<tr>
<td>References</td>
<td><a href="https://www.ejprarediseases.org/">https://www.ejprarediseases.org/</a></td>
</tr>
<tr>
<td>Description</td>
<td>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:</td>
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<tr>
<td>Pillar 0: Coordination, Transversal Activities &amp; Communication</td>
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<tr>
<td>Pillar 1: Fundings and Calls</td>
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<tr>
<td>Pillar 2: Coordinated Access to Data and Services</td>
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</table>
Pillar 3: Training and Empowerment

Pillar 4: Innovation and Clinical Trials Support

WP1 COORDINATION & MANAGEMENT

WP2 STRATEGY
WP3 SUSTAINABILITY
WP4 ETHICS, LEGAL, REGULATORY & IPR
WP5 COMMUNICATION & DISSEMINATION

P1
WP6 Joint Transnational Calls
WP7 Networking scheme
WP8 RDR Challenges
WP9 Monitoring of funded projects

P2
WP10 User-driven strategic planning for P2
WP11 Virtual Platform for data & resources
WP12 Enabling sustainable FAIRness
WP13 Holistic approaches for rare disease diagnostics and therapeutics

P3
WP14 Training on data management & quality
WP15 Capacity building and training of patients and researchers
WP16 Online Academic education course
WP17 ERN RD training and support programme
WP18 Development and adaptation of training activities

P4
WP19 Facilitating partnerships and accelerating translation
WP20 Validation, use and development of innovative methodologies for clinical studies

EJP RD STRUCTURE

COORDINATION & TRANSVERSAL ACTIVITIES

INTEGRATIVE RESEARCH STRATEGY

SUSTAINABILITY

ETHICAL & REGULATORY

COMMUNICATION

1 FUNDING
2 COORDINATED ACCESS
3 CAPACITY BUILDING
4 ACCELERATING TRANSLATION OF RESEARCH & THERAPY DEVELOPMENT

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)
| Availability | European Reference Networks (ERNs) & Non-profit Organizations
|             | Patient Research & Funding
|             | 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

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<th>Best time to apply and time window</th>
<th>The tool has its best use as early as possible and throughout the development process.</th>
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<tbody>
<tr>
<td>Expert tips</td>
<td>PROs:</td>
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<tr>
<td></td>
<td>– Optimization of resources, sustainable programs for research, optimization of infrastructure use, focused and non-redundant research projects. Better programming of research investments.</td>
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<tr>
<td></td>
<td>CONs:</td>
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<tr>
<td></td>
<td>– Optimized coordination with ERNs</td>
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