EUROPEAN JOINT PROGRAMME ON RARE DISEASES

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Paris, France
The overall objective is to implement a European Joint Programme (EJP) Cofund for Rare Diseases which would 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. The initiative should follow the policies and contribute to the objectives of the International Rare Diseases Research Consortium (IRDiRC). The specific objectives of the EJP Cofund are to 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.
<table>
<thead>
<tr>
<th>Date</th>
<th>Event Description</th>
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<tr>
<td>March 2016</td>
<td>First discussions started at E-Rare strategic workshop in Barcelona,</td>
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<td>August 2016</td>
<td>The Commission sends official invitation to MS to nominate experts to the EJP RD working groups</td>
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<td>October 2016</td>
<td>First meeting of the MS EJP RD expert group</td>
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<td>Dec 2016 – Feb 2017</td>
<td>Preparation of the 1st Concept Draft of the EJP RD</td>
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<td>April 2017</td>
<td>2nd meeting of the MS EJP RD experts group</td>
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<td>Apr – June 2017</td>
<td>Nomination of new experts to future WGs</td>
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<td>June 2017</td>
<td>Kick off meeting of the extended WGs of the EJP RD</td>
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<tr>
<td>June – Oct 2017</td>
<td>Preparation of the 2nd Concept Draft of the EJP RD  more than 70 experts involved + 24 ERN coordinators representing EU, associated and non-EU countries</td>
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<td>12 Oct 2017</td>
<td>2nd EJP RD Concept Draft sent to the EC and WGs + ERN Coo</td>
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<td>17 Oct 2017</td>
<td>Last meeting of the MS</td>
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<td>End Oct 2017</td>
<td>Official publication of the WP 2018 – 2020  kick off of the writing phase</td>
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Next-generation data infrastructure for RD (dvpt & MGT)

Multi-omics strategies to unravel new disease genes and mechanisms

Innovative strategies for the dvpt of therapies

Brokerage system
- Joint Transnational Calls
- Networking support scheme
- RD Challenge (PPP collaborations)
- Monitoring of granted projects

- Next generation data infrastructure
- Multi-omics strategies to unravel new disease mechanisms
- Innovative strategies for development of therapies and CTs
- Brokerage system

- Support to accelerate transnational research
  - Guiding service & access to infras
  - Direct assistance to funded projects
- Support to design CTs

- Training on data management & quality
- Capacity building and training of patients
- Online academic education courses
- ERN RD research training and support programmes
PILLAR 1: RESEARCH COLLABORATIVE FUNDING

How ERNs can participate?

• Directly responding to Joint Transnational Calls as research/clinical partners → NOTE: it is not the whole ERN but individual research teams that apply within the proposed project consortia

• Directly responding to Networking Support Scheme → NOTE: cost of research are not supported, only networking

• Directly responding to RD Challenge scheme as academic partners for industry/private sector challenge → NOTE: focus on translation and rapid product development (max. 12 months)
PILLAR 2: COORDINATED ACCESS TO DATA AND SERVICES

How ERNs can participate?

- As USERS by using the platform and providing data
- As DEVELOPERS by contributing to the platform development
- As RESEARCHERS by participating in pilot project for ex. on development of methodologies specific for RD clinical trials
- As INFRASTRUCTURES by providing the resources (samples, patients, experts)
- As CLIENTS in the brokerage system, when looking for validation of genes
PILLAR 3: CAPACITY BUILDING AND EMPOWERMENT

How ERNs can participate?

• As TRAINEES participating in the available training schemes and e-learning
• As PROVIDERS of trainings developed under the specific ERN(s) scheme
• As (CO)DEVELOPERS of new trainings or expansion of already existing ones
PILLAR 4: HELPDESK & INNOVATION SUPPORT SERVICE

How ERNs can participate?

- As USERS of the Helpdesk when coming with specific research questions
- As EXPERTS when participating in expert board and guidance development
- As INFRASTRUCTURES to be involved in the future projects developing therapies or clinical trials
HOW ERNS CAN PARTICIPATE?

Administrative and operational issues

OPTION 1: ALL ERNs are direct beneficiaries of the EJP RD

- Via their COORDINATING partner
- Via SINGLE designated partners (one per ERN)

NOTE:
- Chosen partner must have the capacity to distribute funds to other partners
- ERNs should agree on the process of the ‘ad hoc’ involvement of the ERN partners in different tasks (who participate in what and in which year → this will be translated through annual Work Plans of the EJP RD)
- The tasks in which ERNs will be involved should be pre-identified to justify the participation
- In this option other partners are considered as “third parties” of the direct beneficiary. They need to be named in the EJP (flexibility of involvement) and will have to report their costs to the direct beneficiary each year. The financial reporting to the EC is under responsibility of direct beneficiary
- It is not possible to involve over 300 of ERN partners directly
HOW ERNS CAN PARTICIPATE?

Administrative and operational issues

OPTION 2: Only some ERNs are direct beneficiaries of the EJP RD

• Via IDENTIFIED specific partner(s)

• Inclusion of new ERN institutions is not excluded but must be reported in advance and will be done through the amendment to the GA

NOTE:

• The specific ERN institutions that will be involved from the start of the EJP RD should be identified in advance (during the writing phase) and assigned to specific tasks in the EJP RD → it is up to the ERNs to discuss and agree on the process/identification

• The “new” ERN institutions should be identified whenever relevant and reported as new partners to be involved in the respective annual Work Plan → the planning should be done sufficiently in advance in order to issue the amendment to the GA
THANK YOU

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