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EUROPEAN JOINT PROGRAMME ON RARE DISEASES

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EUROPEAN JOINT PROGRAMME ON RARE DISEASES

Objective (as indicated in the EC WP 2018 – 2020 call text)

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.**

EUROPEAN JOINT PROGRAMME ON RARE DISEASES

Background of preparatory work

- **March 2016** - First discussions started at E-Rare strategic workshop in Barcelona,
- **August 2016** - The Commission sends official invitation to MS to nominate experts to the EJP RD working groups
- **October 2016** - First meeting of the MS EJP RD expert group
- **Dec 2016 – Feb 2017** - Preparation of the 1st Concept Draft of the EJP RD
- **April 2017** - 2nd meeting of the MS EJP RD experts group
- **Apr – June 2017** – nomination of new experts to future WGs
- **June 2017** – kick off meeting of the extended WGs of the EJP RD
- **June – Oct 2017** – 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
- **12 Oct 2017** – 2nd EJP RD Concept Draft sent to the EC and WGs + ERN Coos
- **17 Oct 2017** – last meeting of the MS
- **End Oct 2017** – official publication of the WP 2018 – 2020 → kick off of the writing phase

Coordination & Transversal Activities

Strategic Coordination
& MGT

Communication &
Dissemination

Sustainability

IRDIRC
Secretariat

Pillar 1

Funding Collaborative
Research on RD

- Joint Transnational Calls for collaborative research projects
- Networking to share knowledge on rare diseases
- Rare Disease Research Challenges
- Monitoring of granted projects

Pillar 2

Coordinated access to
RD data and services

- 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

Pillar 3

Capacity building and
empowerment

- Training on data MGT & quality
- Capacity building & Training of Patients
- Online Academic education course
- ERN RD research training and support programmes

Support to design of clinical trials

Support to accelerate translational research

Pillar 4 : Helpdesk & Innovation support service

FUNDING

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

1

COORDINATED ACCESS

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

2

COORDINATION & TRANSVERSAL ACTIVITIES

4

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

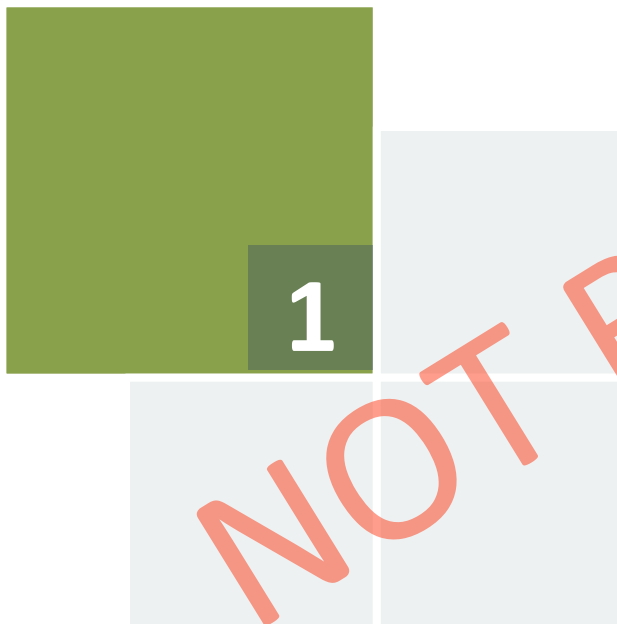
HELPDESK & INNOVATION

3

- Training on data management & quality
- Capacity building and training of patients
- Online academic education courses
- ERN RD research training and support programmes

CAPACITY BUILDING

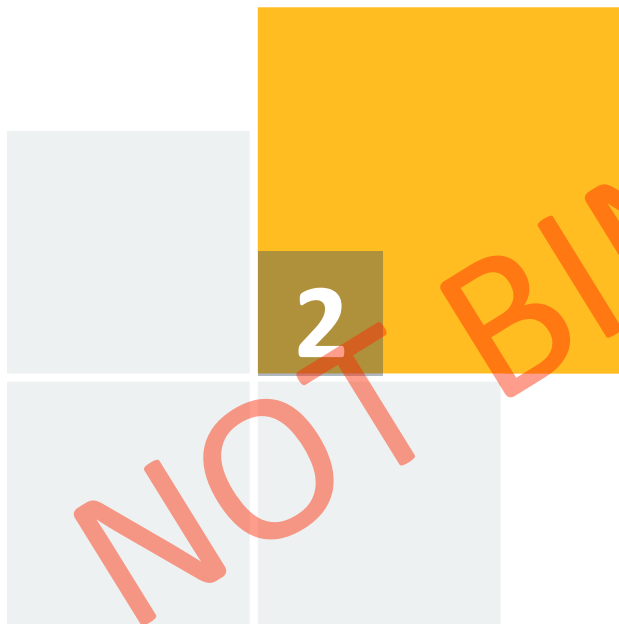
PILLAR 1: RESEARCH COLLABORATIVE FUNDING



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)

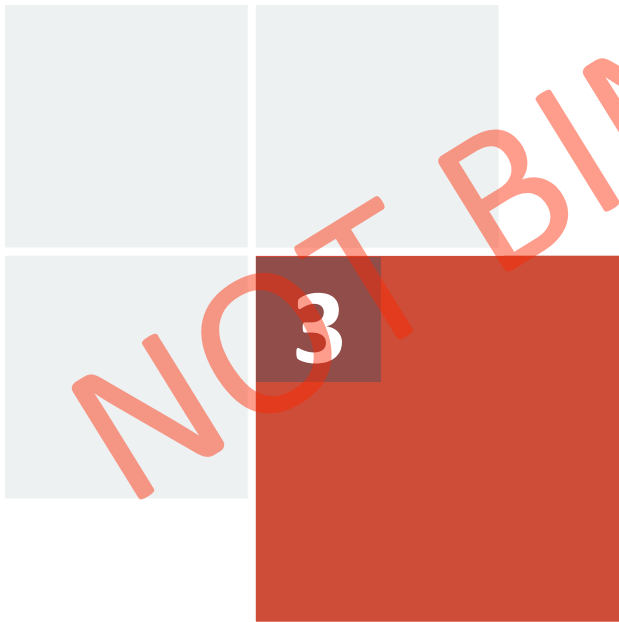
PILLAR 2: COORDINATED ACCESS TO DATA AND SERVICES



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



4

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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