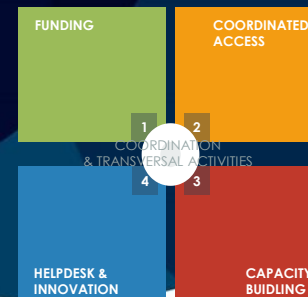


Le projet de programme conjoint européen sur les Maladies Rares

EJP Rare diseases

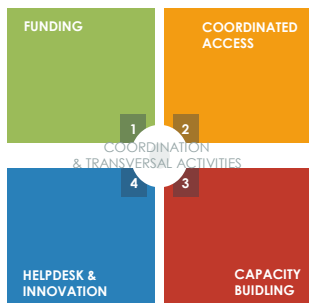


Info Day MESRI 10 novembre 2017

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MINISTÈRE
DE L'ÉDUCATION
NATIONALE, DE
L'ENSEIGNEMENT
SUPÉRIEUR ET DE
LA RECHERCHE



EUROPEAN JOINT PROGRAMME ON RARE DISEASES

OBJECTIVES

- **Main objective:** 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
- **Specific objective:** 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 an efficient model of financial support for research on rare diseases

- **Union contribution:** 55 M€
- **Total budget (min):** 78,5 M€ (→ expected > 110 M€)
- **Foreseen number of partners:** 70 – 100
- **Foreseen number of participating countries:** 29 including 23 EU MS, 4 associated (CH, IL, NO, TK) and 4 third countries (AU, CA, JP, USA)

EJP RD STRUCTURE (TYPE OF ACTIVITIES)

FUNDING

Financial support to third parties:

- Joint Transnational Calls
- Networking support scheme
- RD Challenge (PPP collaborations)

Monitoring of granted projects

1

COORDINATED ACCESS

Direct research activities:

- Next generation data infrastructure
- Multi-omics strategies to unravel new disease mechanisms (internal calls)
- Innovative strategies for development of therapies and CTs (internal calls)
- Small brokerage system (support to 3rd parties)

2

COORDINATION
& TRANSVERSAL ACTIVITIES

4

- Support to accelerate translational 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

TRAINING & CAPACITY BUILDING

COLLABORATION WITH INDUSTRIES

COLLABORATION WITH PATIENT ORGS



EJP RD (TYPE OF PARTICIPATING ORGANIZATIONS)

FUNDING

- Research funding organizations (RFO) public (national agencies) & private (foundations and charities)
- In collaboration (NOT direct beneficiaries) with private sponsors (pharmaceutical companies, venture capital, etc.)

1

COORDINATED ACCESS

- Research Performing Organizations (RPO): research institutes, universities, private institutes and foundations
- European Research Infrastructures
- European Reference Networks

2

COORDINATION & TRANSVERSAL ACTIVITIES

4

- Research Performing Organizations (RPO): research institutes, universities, private institutes and foundations
- European Reference Networks
- European Research Infrastructures
- In collaboration (NOT direct beneficiaries) with private sponsors (pharmaceutical companies, venture capital, etc.)

HELPDESK & INNOVATION

3

- Research Performing Organizations (RPO): research institutes, universities, private institutes and foundations
- European Research Infrastructures
- EURORDIS (ONG)
- European Reference Networks

TRAINING & CAPACITY BUILDING

Opportunités et Défis l'EJP Maladies Rares

- **Opportunités**

- Création d'un écosystème pérenne pour les maladies rares, permettant l'établissement d'un cercle vertueux entre le soin la recherche et l'innovation
- Accélérer le diagnostic et le développement de nouveaux traitements pour les malades en accord avec la vision d'IRDiRC
- Meilleures utilisations des ressources et initiatives financées
- Forte visibilité pour les Maladies Rares au travers de l'implication des ministères de la Santé et de la Recherche des EM
- Placer l'Europe en position de leader de la R&D des Maladies Rares

- **Défis**

- Identification les partenaires très variés de l'EJP
- Assurer une plus-value pratique pour les chercheurs, soignants et malades
- Assurer une participation large des pays y compris des plus petits
- Identifier des sources supplémentaires de financement de la recherche au niveau national et européen (FP9).

Calendrier

06/04 – Discussion sur EJP RD au comité de programme Santé H2020

18/04 – Meeting du group miroir français de l'EJP RD au MENESR

25/04 – Meeting du groupe d'experts Maladies Rares nommés par les pays → premières discussions sur le contenu de l'EJP MR

May 2017 – Adoption du Programme de travail 2018-2020 du comité de programme Santé H2020

Nov 2017 – Ouverture de l'appel à projet EJP RD

April 2018 – Soumission de la proposition pour l'EJP MR

HOW TO CONTRIBUTE TO THE EJP RD?

FUNDING

1

COORDINATED ACCESS

2

COORDINATION
& TRANSVERSAL ACTIVITIES

4

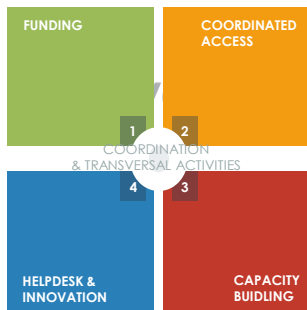
HELPDESK & INNOVATION

3

CAPACITY BUILDING

IDENTIFICATION OF NEW PARTNERS

NEXT STEPS



PROCESS & TIMELINE

- Each country has the possibility to propose new institutions
- The proposed institutions should comply with specific criteria (overall or for designated pillar)
- Proposed deadline to complete the process: 17 November 2017

It is necessary to limit the number of EJP RD partners for the sake of « managability » of the whole programme. Therefore:

- It is proposed to limit the number of NATIONAL institutions to 3
- This rule does not apply to:
 - EU/international institutions having the central office in specific countries (EU infras, ERNs, EURORDIS, etc.)
 - National/regional funding bodies as long as they are Programme Owners or endorsed Programme Managers

IDENTIFICATION OF NEW PARTNERS

PROPOSED CRITERIA



MAJOR CRITERION FOR ALL PILLARS

- Proposed partner should be nationally/internationally recognized for its excellence and relevance to the specific RD activity
- Proposed partner should have capacity to contribute to the EJP RD (by providing in kind or in cash contributions; services; expertise, etc.)

PILLAR 1 CRITERIA

- Proposed partner should have capacity to finance research through open calls
- Proposed partner should be able to agree on rules established for transnational joint funding: ex. validate and respect results of transnational peer review

IDENTIFICATION OF NEW PARTNERS

PROPOSED CRITERIA



MAJOR CRITERION FOR ALL PILLARS

- Proposed partner should be nationally/internationally recognized for its excellence and relevance to the specific RD activity
- Proposed partner should have capacity to contribute to the EJP RD (by providing in kind or in cash contributions; services; expertise, etc.)

PILLAR 2 CRITERIA

- Proposed partner should provide wide-used standards, methods, models or tools having achieved proven results and that can be applicable to RD. These can cover diagnosis, phenotyping, data collection, storage and analysis, cell/animal models, biomarkers and outcomes measures validation.
- Proposed partner should provide unavoidable collections of samples, clinical, biological or imagery data on RD.

IDENTIFICATION OF NEW PARTNERS

PROPOSED CRITERIA



MAJOR CRITERION FOR ALL PILLARS

- Proposed partner should be nationally/internationally recognized for its excellence and relevance to the specific RD activity
- Proposed partner should have capacity to contribute to the EJP RD (by providing in kind or in cash contributions; services; expertise, etc.)

PILLAR 3 CRITERIA

- Proposed partner should be a source of (nationally established) transversal training (in English) relevant to rare diseases. The proposed training should not be covered or should be complementary to the already pre-identified trainings
- OR
- Proposed partner should have capacity to provide training at national or international level

IDENTIFICATION OF NEW PARTNERS

PROPOSED CRITERIA



MAJOR CRITERION FOR ALL PILLARS

- Proposed partner should be nationally/internationally recognized for its excellence and relevance to the specific RD activity
- Proposed partner should have capacity to contribute to the EJP RD (by providing in kind or in cash contributions; services; expertise, etc.)

PILLAR 4 CRITERIA

- Proposed partner should have already established capacity to perform RD clinical trials or develop methodologies for RD clinical trials
- OR
- Proposed partner should have (already proven) capacity to act as facilitator in the process of research translation (methodological, legal/regulatory, establishment of partnerships with private sector). Such service should be available at least at national level. The type of client(s) should be specified

MERCI

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