Le projet de programme conjoint européen sur les Maladies Rares

EJP Rare diseases

Info Day  MESRI 10 novembre 2017

www.enseignementsup-recherche.gouv.fr
**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

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)

COORDINATION & TRANSVERSAL ACTIVITIES

• Support to accelerate translational research
  o Guiding service & access to infras
  o Direct assistance to funded projects
• Support to design CTs

HELPDESK & INNOVATION

• 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
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.)

COORDINATED ACCESS

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

COORDINATION & TRANSVERSAL ACTIVITIES

1. FUNDING
2. COORDINATED ACCESS
3. TRAINING & CAPACITY BUILDING
4. HELPDESK & INNOVATION

- 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.)
- EURORDIS (ONG)
- European Reference Networks
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
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
MAJOR CRITERION FOR ALL PILLARS

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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.
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IDENTIFICATION OF NEW PARTNERS
Proposed criteria

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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

POUR TOUTE QUESTION CONTACTER DARIA JULKOWSKA
DARIA.JULKOWSKA@AGENCERECHERCHE.FR