CURRENT FUNDING (and support) SCHEMES

European Joint Programme on Rare Diseases & Horizon Europe

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European Joint Programme on Rare Diseases
INSERM, France

Support for development of orphan medicines

Incentives, scientific advice and funding schemes

European Medicines Agency, 30 November 2020

Objectives of the EJP RD

- **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

- **Mode of action:**

Large programme that integrates existing infrastructures, trainings, funding programmes and tools, expands them and develops new essential ones to offer harmonized (and centralized) RD research ecosystem that is easy to use for scientists and produces benefits for patients in the most efficient way

EJP RD

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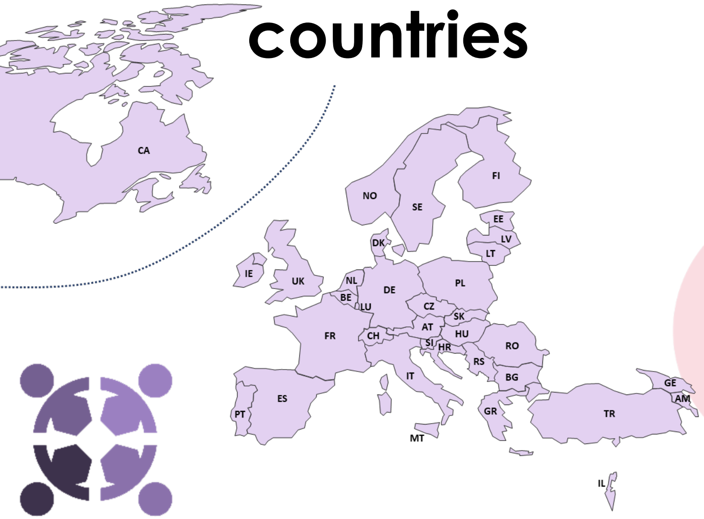
European Union
GA n°825575

85% of
European RD community
(directly or indirectly)
involved in **EJP RD**

750 people:

650 Scientifics
100 Admin

35
participating
countries



88 Beneficiaries:

9 hospitals
12 research institutes
31 research funding bodies/ministries
24 universities/hospital universities
5 EU infrastructures
5 charities/foundations
EURORDIS

+

50
Linked Third Parties

And 100% of the associated networks

EURORDIS:
884 RD patient organisations
72 countries

EATRIS
13 main national nodes

INFRAFRONTIER
23 partners
15 countries

24 ERNs:
300 institutions
>950 healthcare units
26 countries

ECRIN
12 main national nodes

ELIXIR
220 research organisation
23 partners

BBMRI
1 international partner
21 main national nodes
20 countries

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EJP RD STRUCTURE

Coordinated by

Inserm

La science pour la santé
From science to health

COORDINATION
& TRANSVERSAL ACTIVITIES

INTEGRATIVE RESEARCH STRATEGY

SUSTAINABILITY

ETHICAL & REGULATORY

COMMUNICATION

1 FUNDING

2 COORDINATED ACCESS TO DATA & SERVICES

3 CAPACITY BUILDING & EMPOWERMENT

4 ACCELERATING TRANSLATION OF RESEARCH & THERAPY DEVELOPMENT

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EJP RD
-
what is there for me?

FUNDING, TRAINING & SUPPORT OFFICES

EUROPEAN JOINT PROGRAMME RARE DISEASES

JOIN TRANSNATIONAL CALLS

Joint Transnational Calls

- **MAIN GOAL:** enable scientists in different countries to build an effective collaboration on a common interdisciplinary research project based on complementarities and sharing of expertise, with a clear future benefit for patients
- **MAIN CHARACTERISTICS (some minor modifications may apply every year):**
  - Launched every year in December, pre-announcement in November
  - 2-stage evaluation process (short pre-proposals + invitation to submit full proposal after 1st round of scientific evaluation)
  - Pre-proposal submission stage open for 60 days
  - Rebuttal stage included in full proposal evaluation (applicants have possibility to respond to evaluators' comments)
  - A minimum of 3 eligible research teams and a max. of 6 per project (can be extended to 8 according to specific conditions)
  - Involvement of under-represented countries is encouraged
  - Involvement of Patient Advocacy Organisations is encouraged
  - Projects are multinational but funding is national (contract is signed by national funding bodies)
  - Typical success rate: 1st stage vs final funding = 10-12%; 2nd stage vs final funding 35 -50%

MORE INFORMATION: https://www.ejprarediseases.org/index.php/fundings-and-calls/

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Joint Transnational Call 2021

Objective: support transnational, innovative, and interdisciplinary Social Sciences and Humanities research projects to improve healthcare implementation and everyday life of people living with a rare disease

List of topics:

- **Health & social care services** research to improve patient and familial/household health outcomes
- **Economic Impact of Rare diseases**
- **Psychological and Social Impact of Rare diseases**
- Studies addressing **the impact/burden of the delay in diagnosis and of the lack of therapeutic intervention.**
- **e-Health in rare diseases**: Use of innovative technology systems for care practices in health and social services
- Development and enhancement of **health outcomes research methods** in rare diseases
- Effects of **pandemic crisis** and the **global outbreak alert** and response on the rare disease field, and the emergence of innovative care pathways in this regard.

Other research topics are possible as long as they focus on SSH research and are not in the excluded topics list.

The call is scheduled to open in December 2020
with a pre-proposal submission deadline in February 2021

EJP RD

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EJP RD internal call for projects on innovative statistical methodologies to improve RD clinical trials in limited populations

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RARE DISEASES

EJP RD internal call for projects on innovative statistical methodologies to improve RD clinical trials in limited populations

- Aims to develop innovative statistical methodologies to address unmet needs associated the development and the analysis of clinical trials in limited populations
- Submission of projects is limited to partners from institutions beneficiaries of the EJP RD. This **includes all 24 ERNs** and Linked Third Parties or, parties bound by the Network Agreement with the beneficiary institution (and thus being able to integrate EJP RD project as Linked Third Party at later stage)
- **Opening of the call is foreseen in December 2020**
- A networking/matchmaking event will be organised on 11 of January 2021

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Are you looking for (e.g):

Gathering of experts & patients to discuss and share knowledge?

Expanding your network to include new stakeholders?

Finding ways to gather and support future consortium that plans to apply to EC calls?

EUROPEAN JOINT PROGRAMME
RARE DISEASES

Networking Support Scheme

- **Objective:** encourage sharing of knowledge on rare diseases and rare cancers
  - to support health care professionals, researchers and patient advocacy organizations with a networking grant to re-organize themselves into transnational (clinical) research networks
  - that focus on a (group of) rare disease(s), a (group of) rare cancer(s) or on cohorts of undiagnosed patients that are suspected of suffering from a rare disease
- **Financial support to applicants for fostering organization of workshops or conferences for new research networks or existing/expanding research networks to strengthen collaborations and to enable exchange of knowledge**
- 30K€ max per event
- **Applications:**
  - open on a continuous basis. The applications will be collected every three months and the eligibility will be checked. First collection of application: **December 1, 2020 at 14:00 (CET)**
  - open to all countries involved in EJP RD (Applicants from Canada are not eligible for funding)
  - the consortium submitting an application must involve a **minimum of three eligible applicants** from **at least three different countries** participating in the EJP RD at the time of the application. A **maximum of 10 partners** per application is eligible

MORE INFORMATION: http://www.ejprarediseases.org/index.php/networking-support/

EJP RD

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Are you looking to:

Train your PhD student/young MD within your ERN network or within other ERN network?

Research Mobility Fellowship

- **Aim:** financially support **PhD students** and **medical doctors** working in **ERN-member institutions** to undertake **short scientific visits** (secondments) up to 3 months fostering specialist research training outside their countries of residence and within one of the ERN host institutions. Through this training measure the fellows should acquire at their host (secondment) institution new competences and knowledge related to their research on rare diseases and with benefit to their ERN.

- **Applicants/Application profile**:
  - PhD students with a minimum of one year of research experience OR physicians having finished their first year of specialist training
  - Be affiliated to an **ERN Full Member or to an ERN-Affiliated Partner Institution** from one of the 24 ERNs at the time when the application is submitted, as well as during the proposed period of the training stay
  - The host (secondment) institutions must be Full or Affiliated Members of an ERN at the time when the application is submitted, as well as during the proposed period of the training stay
  - Added value to ERN of the mobility stay

The call opened on October 1st 2020

Deadline for submission: 13th November 2020

MORE INFORMATION: https://www.ejprarediseases.org/index.php/training-and-empowerment/ern-trainings/

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Are you looking to:

Share transversal type of knowledge (going beyond your ERN) and have interesting training idea?

Research Training Workshop

- **Aim:** identify the most suitable proposals for the **organization of research training workshops of 2 days targeted to the ERNs needs.** Selected research training workshops will have to train ERN researchers and clinicians in ERN relevant innovative training themes. Training themes may include innovative research methodologies, diagnostic research methodologies, interdisciplinary treatment approaches, such as gene therapy and transplantation, etc. Moreover, the workshops will be aiming to provide a cross-ERN added value.

  The workshops will be implemented as two day events. Topics can be proposed by the ERNs or by investigators belonging to EJP RD beneficiary institutions.

- **25k€ max/event**
- **Applicants/Application profile**:
  - Affiliated to any EJP RD beneficiary institution
  - Affiliated to an ERN Full Member
  - Affiliated to an ERN Partner institution at the time when the application is submitted, as well as during the period of the execution of the workshop

2 calls per year - the call closed on October 12 2020
Next call will open in Q1 2021

MORE INFORMATION: https://www.ejprarediseases.org/index.php/training-and-empowerment/ern-trainings/

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Are you looking to:

Train your members or gain knowledge in rare diseases research related aspect?

EUROPEAN JOINT PROGRAMME
RARE DISEASES

Other EJP RD trainings

- **Data management & quality:** the training activities cover a variety of topics including but not limited to *variant interpretation, data management, registries, FAIRification process, Orphacodes, biobanking, and undiagnosed cases.*
- **Patients & Researchers training:** training modules dedicated to building the capacity of the patient community and other key stakeholders, including training "expert" paediatric patients (new in 2021) on rare diseases, paediatric medicines development and clinical research.
- **Educational academic course:** on transversal and multidisciplinary aspects of rare diseases research will be made available for all stakeholders in a fully online format. The course foresees the development of 5 modules. The implementation of the first modules are expected by the end of 2020.

MORE INFORMATION: https://www.ejprarediseases.org/index.php/training-and-empowerment/

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You have project and/or preliminary results for e.g. new therapy/biomarker/device

&

You are looking for (e.g):

- *evaluation of translational potential of your project*
- *further regulatory support*
- *advice on how to get interest of potential sponsor or derisk your project*

Support for innovation & research translation

- **Facilitating partnerships and accelerating translation for higher patient impact**

Innovation management toolbox

- Integration of various resources supporting research translation (including the IRDiRC Orphan Drug Dev. Guide)
- Connection with Pillar 2 to make it interoperable with the Virtual Platform

Support for translation & innovation mentoring

- **Pool of mentors to support the translation & innovation** of research projects
- **Assessment of the translation needs** (primary open to E-Rare, EJP RD funded projects and ERN projects but other projects can also benefit based on capacity of the service)

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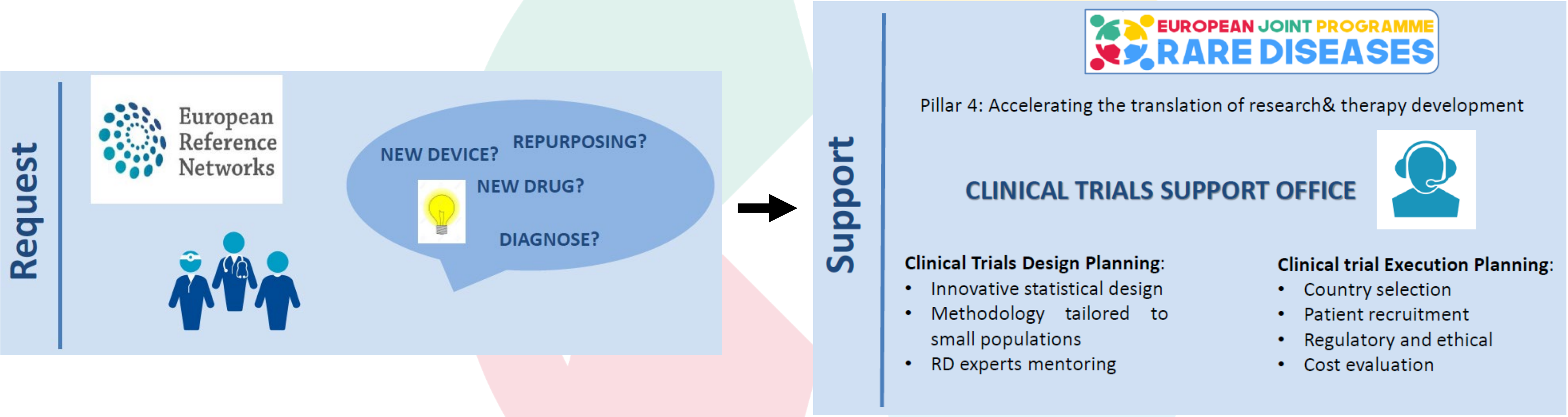
You have idea about possible multinational clinical study but for which e.g. there is no interest from industry?

&

You are looking for (e.g):

- *advice on how to advance with such project*
- *advice on how to put in place clinical study with public sponsors*
- *advice on overall management & regulatory issues of multinational clinical studies*

Multinational CTs support office



Request

European Reference Networks

NEW DEVICE?

REPURPOSING?

NEW DRUG?

DIAGNOSE?

Support

EUROPEAN JOINT PROGRAMME RARE DISEASES

Pillar 4: Accelerating the translation of research& therapy development

CLINICAL TRIALS SUPPORT OFFICE

Clinical Trials Design Planning:
- Innovative statistical design
- Methodology tailored to small populations
- RD experts mentoring

Clinical trial Execution Planning:
- Country selection
- Patient recruitment
- Regulatory and ethical
- Cost evaluation

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We all work for the benefit of rare diseases patients
& EJP RD is here to support YOU!

CONTACT US

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helpdesk@ejprarediseases.org

EUROPEAN JOINT PROGRAMME
RARE DISEASES

FUNDING OPPORTUNITIES UNDER HORIZON EUROPE

Preliminary structure of Horizon Europe

Pillar 1
Excellent Science

- European Research Council
- Marie Skłodowska-Curie Actions
- Research Infrastructures

Pillar 2
Global Challenges and European Industrial Competitiveness

Clusters
- Health
- Culture, Creativity and Inclusive Society
- Civil Security for Society
- Digital, Industry and Space
- Climate, Energy and Mobility
- Food, Bioeconomy, Natural Resources, Agriculture and Environment

Joint Research Centre

Pillar 3
Innovative Europe

- European Innovation Council
- European innovation ecosystems
- European Institute of Innovation and Technology

Widening Participation and Strengthening the European Research Area

- Widening participation and spreading excellence
- Reforming and Enhancing the European R&I system

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Marie-Sklodowska Curie Actions

(information based on Horizon 2020 scheme)

- **Bottom up (no specific RD topic focus)**
- MSCA Individual Fellowships → for experienced **researchers** looking to give a boost to their career by working abroad. Profit both researcher & **host laboratory**. Focus on specific research project (including training).
- MSCA Cofund: Co-funding of regional, national and international programmes → provides **organisations** with additional financial support for their own researcher training and career development programmes. Supports doctoral programmes for PhD candidates & fellowship programmes for experienced researchers.
- MSCA ITNs: Innovative Training Networks → bring together **universities/research institutes & other sectors** from accross the world to train researchers to doctorate level.
- MSCA RISE: Research and Innovation Staff Exchange → short-term exchanges (1 month to 1 year) of **personnel** between academic, industrial and commercial organisations throughout the world. 3 partners from 3 different countries (2 among EU or associates MS). Exchange must be between **organisations** of different sectors.

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Horizon Europe: Health Work Programme

(information not binding)

- **GENERAL CHARACTERISTICS:**
  - **Keywords to keep in mind: social rights, economy (affordable and innovative healthcare), missions (cancer plan, green deal), one health approach, fit for digital age**
  - « Top-down » approach: the topics are pre-defined
  - The first work programme is defined for the period 2021-2022
  - Calls include 1 or 2-step application/evaluation procedure
  - Call include different type of actions:
    - RIA: research & innovation action
    - CSA: coordination & support action
    - Partnership
    - PCP: pre-commercial procurement
    - PPI: public procurement for innovative solutions (market ready, market uptake)
  - Rare diseases specificity:
    - Some topics may be RD–specific, e.g. development of effective therapies for rare disease with an unmet medical needs
    - The majority will be non-RD specific but RD community will be able to apply (except if specific mention applies, e.g. focus on disorders with high prevalence and burden)

https://ec.europa.eu/info/funding-tenders/opportunities/portal/screen/home

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EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

THANK YOU

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RARE DISEASES