



# European Joint Programme on Rare Diseases (EJP RD)

## Call for Proposals 2020

### "PRE-CLINICAL RESEARCH TO DEVELOP EFFECTIVE THERAPIES FOR RARE DISEASES"

#### Preliminary Announcement

**The content and procedures of the call described in this pre-announcement may be subject to change and are not legally binding.**

The ERA-Net E-Rare has successfully implemented ten Joint Transnational Calls for rare disease research projects since 2006. This effort continues in the framework of the **European Joint Programme on Rare Diseases (EJP RD)** that has been established to further help in coordinating the research efforts of European, Associated and non-European countries in the field of rare diseases and implement the objectives of the International Rare Disease Research Consortium (IRDiRC).

A number of national and regional funding organisations will participate in the **EJP RD Joint Transnational Call (JTC) 2020** and will fund multilateral research projects on rare diseases **together with the European Commission (EC) under the EJP-COFUND action**. The call opens simultaneously with the involvement of the following funding organisations in their respective countries/regions:

- Austrian Science Fund (FWF), Austria
- Research Foundation Flanders (FWO), Belgium, Flanders
- Fund for Scientific Research - FNRS (F.R.S.-FNRS), Belgium, French-speaking community
- Canadian Institutes of Health Research – Institute of Genetics (CIHR-IG), Canada
- Fonds de recherche du Québec-Santé (FRQS), Québec (Canada)
- Ministry of Education, Youth and Sports (MEYS), Czech Republic
- Academy of Finland (AKA), Finland
- French National Research Agency (ANR), France
- French Foundation for Rare Diseases (FFRD), France
- INSERM, France (Patient Advocacy Organisations)
- **Decision Pending:** Federal Ministry of Education and Research (BMBF), Germany



- **Decision Pending:** German Research Foundation (DFG), Germany
- General Secretariat for Research and Technology (GSRT), , Ministry of Development & Investments, Greece
- National Research, Development and Innovation Office (NKFIH), Hungary
- Health Research Board, (HRB), Ireland
- Chief Scientist Office of the Ministry of Health (CSO-MOH), Israel
- Italian Ministry of Health (MoH-IT), Italy
- Ministry of Education, Universities and Research (MIUR), Italy
- Fondazione Regionale per la Ricerca Biomedica (FRRB) Lombardy, Italy
- Tuscany Region (RT/TuscReg), Tuscany (Italy)
- Research Council of Lithuania (RCL), Lithuania
- National Research Fund (FNR), Luxembourg
- National Centre for Research and Development (NCBR), Poland
- **Decision Pending:** The Foundation for Science and Technology (FCT), Portugal
- Slovak Academy of Sciences (SAS), Slovakia
- National Institute of Health Carlos III (ISCIII), Spain
- Swedish Research Council (SRC), Sweden
- Swiss National Science Foundation (SNSF), Switzerland
- Netherlands Organization for Health Research and Development (ZonMw), The Netherlands
- The Scientific and Technological Research Council of Turkey (TUBITAK), Turkey

## 1. AIM OF THE CALL

The aim of the call is to 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.

### Topic: PRE-CLINICAL RESEARCH TO DEVELOP EFFECTIVE THERAPIES FOR RARE DISEASES

**Research proposals must cover at least one of the following areas:**

1. Development of novel therapies in a preclinical setting (including small molecules, repurposing drugs, cell and gene advanced therapies) focusing on condition(s) with unmet medical needs
2. Use of disease models suitable for medicinal product's development according to
3. Development of predictive and pharmacodynamics (PD) biomarkers (with appropriate analytical methods e.g. OMICS) in a preclinical setting (e.g. in the validated model or in pre-collected human samples) for monitoring the efficiency of the therapy. The model chosen must mimic the human diseases and be transposable so that the biomarker identified in animals can be valid for humans
4. Proof of principle studies fostering an early (pre-clinical) stage of drug development (excluding interventional clinical trials of phase 1-4).



**The following approaches and topics are excluded from the scope of the call:**

- a) Therapeutic approaches concerning rare infectious diseases, rare cancers and rare adverse drug events in treatments of common diseases
- b) Interventional clinical trials
- c) Surgery or radiation therapies
- d) Studies that focus on research to accelerate diagnosis or to set up new registry/cohort studies to explore disease progression and mechanisms as these were the focus of JTC 2019.
- e) Rare neurodegenerative diseases which are within the main focus of the Joint Programming Initiative on Neurodegenerative Disease Research (JPND; <http://www.neurodegenerationresearch.eu/>). These are: Alzheimer's disease and other dementias; Parkinson's disease (PD) and PD-related disorders; Prion disease; Motor Neuron Diseases; Huntington's disease; Spinal Muscular Atrophy and dominant forms of Spinocerebellar Ataxia. Interested researchers should refer to the relevant JPND calls.

**Childhood dementias/neurodegenerative diseases are not excluded.**

### **Consortium Makeup**

Projects shall involve **a group of rare diseases or a single rare disease following the European definition** i.e. a disease affecting not more than **five in 10.000 persons** in the European Community, EC associated states and Canada. Applicants are encouraged to assemble groups of rare diseases based on solid criteria and commonalities if this leverages added value in sharing resources or expertise and has the capacity to elucidate common disease mechanisms and therapeutic targets.

Partners belonging to one of the following categories may request funding under a joint research proposal (according to country/regional regulations):

- academia (research teams working in universities, other higher education institutions or research institutes),
- clinical/public health sector (research teams working in hospitals/public health and/or other health care settings and health organisations),
- enterprises (all sizes of private companies). Participation of small and medium-sized enterprises (SMEs) is encouraged when allowed by national/regional regulations,
- patient advocacy organisations (PAOs are eligible to obtain funding for their participation in research projects).

**The maximum duration of the project is three years.**

Only transnational projects will be funded. Each consortium submitting a proposal must involve **four to six eligible** principal investigator partners. **No more than two eligible partners from the same country can be present in each consortium** (further



national limits may apply). This limit also applies to Early Career Researchers and partners from underrepresented countries (see below). PAOs requesting funding do not count toward this total.

**The number of partners can be increased to 8 in two cases:**

- The inclusion of partners from participating countries usually underrepresented in projects (Czech Republic, Slovakia, Hungary, Lithuania, Poland, and Turkey).
- The inclusion of Early Career Researchers as full partners.

### 3. PRELIMINARY TIMETABLE

There will be a **two-stage submission procedure** for joint applications: pre-proposals and full proposals. The call is scheduled to open on **December 18<sup>th</sup>, 2019**.

18 <sup>th</sup> February 2020	Registration deadline
18 <sup>th</sup> February 2020	Pre-proposal submission deadline
end April 2020	Invitation to full proposal
16 <sup>th</sup> June 2020	Full proposal submission deadline
28 <sup>th</sup> July 2020	Deadline for rebuttals
October 2020	Notification of funding decision

An independent international Scientific Evaluation Committee will carry out a scientific evaluation according to specific evaluation criteria. Based on this central evaluation, selected consortia will be invited to submit a full proposal by late **April 2020** (deadline for full proposals: **June 16<sup>th</sup>, 2020**).



Further information and the official publication of the call will soon be available on the website of the EJP RD

<http://www.ejprarediseases.org>

For general questions regarding the joint call please contact the Joint Call Secretariat at the ANR, France:

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**For questions regarding national eligibility criteria and requirements please contact the national contact person listed below**

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#### 4. NATIONAL CONTACT POINTS

Country/ Region	Funding Organisation	Contact Details
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