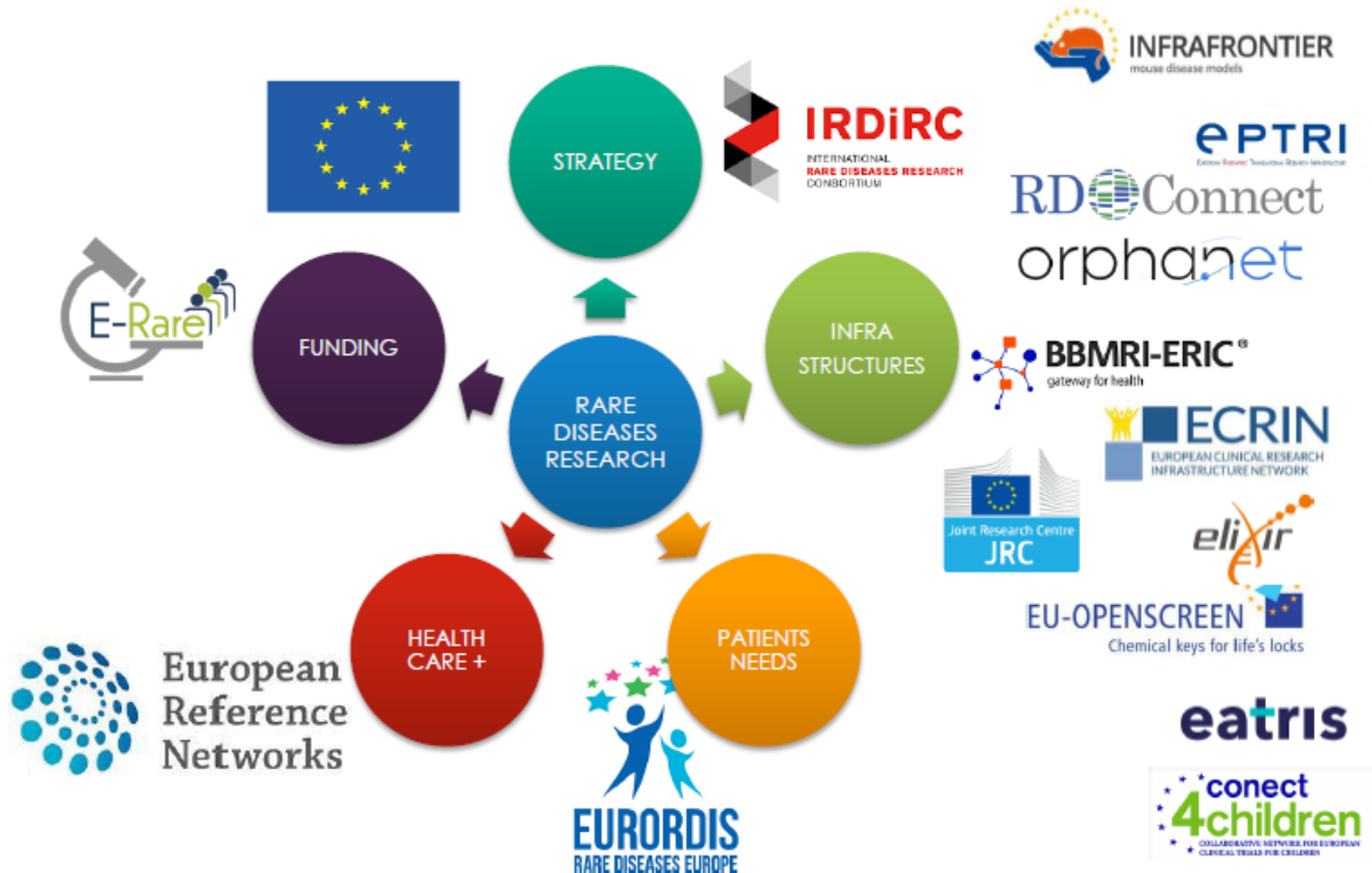


EUROPEAN JOINT PROGRAMME ON RARE DISEASES

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RARE DISEASES LANDSCAPE IN EUROPE



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



EJP RD STRUCTURE

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



WP1 COORDINATION & MANAGEMENT

WP2
STRATEGY

WP3
SUSTAINABILITY

WP4
ETHICS, LEGAL, REGULATORY & IPR

WP5
COMMUNICATION & DISSEMINATION

P1

WP6
Joint Transnational Calls

WP7
Networking scheme

WP8
RDR Challenges

WP9
Monitoring of funded projects

P2

WP 10
User-driven strategic planning for P2

WP 11
Virtual Platform for data & resources

WP 12
Enabling sustainable FAIRness

WP 13
Holistic approaches for rare disease diagnostics and therapeutics

P3

WP 14
Training on data management & quality

WP 15
Capacity building and training of patients and researchers

WP 16
Online Academic education course

WP 17
ERN RD training and support programme

WP 18
Development and adaptation of training activities

P4

WP 19
Facilitating partnerships and accelerating translation

WP 20
Validation, use and development of innovative methodologies for clinical studies

Coordinated by



EUROPEAN JOINT PROGRAMME ON RARE DISEASES

- **Union contribution:** 55 M€ (70% reimbursement rate)
- **Total budget (min. submitted):** 101 M€ (→ expected > 110 M€)
- **Number of partners:** 88
- **Number of participating countries(beneficiaries and LTPs):** 35 including 27 EU MS (AT, BE, BG, CZ, DE, ES, EE, FI, FR, GR, HU, HR, IE, IT, NL, LT, LV, LU, MT, PL, PT, RO, SE, SK, SL, SV, UK), 7 associated (AM, CH, GE, IL, NO, RS TK) and third countries (CA)
- **Timeline:** Jan 2019 – Dec 2023

- **Types of partners:**
 - 31 research funding bodies/ministries
 - 12 research institutes
 - 22 universities/hospital universities
 - 11 hospitals
 - 5 EU infrastructures (BBMRI, EATRIS, ECRIN, ELIXIR, INFRAFRONTIER) + EORTC
 - EURORDIS & ePAGs
 - 5 charities/foundations (FTELE, AFM, FFRD, FGB, BSF)
- } **24 ERNs**

JOINT TRANSNATIONAL CALL

2019

FLORENCE GUILLOT

ANR, FRANCE



WP6: Joint Transnational Calls for collaborative research projects

1st Joint Transnational Call (2019)

- Total budget 27 M€ from participating Member States + min 5 M€ of EC contribution
- 31 funders from 23 countries (AT, BE, CA, CZ, EE, FI, FR, DE, GR, HU, IE, IL, IT, LT, LU, PL, PO, SK, ES, SE, CH, NL, TK)
- Topic: Research projects to accelerate diagnosis and/or explore disease progression and mechanisms of rare diseases

- Research to accelerate diagnosis, e.g:

- New schemes for finding diagnosis for undiagnosed patients;
- Improved annotation and interpretation of variants and development of diagnostic tests for the more prevalent variants;
- Novel modalities of functional analysis of candidate variants through in vitro, cell, tissue or animal studies.
- -omic or multi-omic integrated approaches for discovery of disease causes and mechanisms including development of relevant bioinformatic tools;

- Research to explore disease progression and mechanisms, e.g:

- Natural history studies and patient registries (also for clinical trial readiness). Whenever possible these should include development and use of patient reported outcome measures. In addition, the exploration of the use of standardized M-Health-based surveillance instruments and of patient entered data to gather information for natural history studies is welcome;
- Identification of clinical biomarkers, clinical outcome measures and surrogate endpoints;
- Identification of novel pathophysiological pathways in appropriate disease models that effectively mimic the human condition



WP6: Joint Transnational Calls for collaborative research projects

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

- Approaches concerning rare infectious diseases or rare cancers;
- Approaches concerning rare adverse drug events/medical complications in treatments of common diseases;
- Studies that focus on pre-clinical therapy development and/or validation in cellular or animal models. These will be addressed in future calls;
- Interventional clinical trials;
- Rare neurodegenerative diseases which are within the main focus of the Joint Programming Initiative on Neurodegenerative Disease Research (JPND; <http://www.neurodegenerationresearch.eu/>). These concern: 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. Not excluded through this specification are childhood dementias/neurodegenerative diseases.

FOR OTHER DETAILS (ELIGIBILITY RULES PLEASE CHECK CAREFULLY THE CALL TEXT, GUIDELINES FOR APPLICANTS AND PROPOSAL FORMS)



WP6: Joint Transnational Calls for collaborative research projects

Eligible partners (check the national rules!):

- 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)
- enterprise (all sizes of private companies). Participation of small and medium-size enterprises (SMEs) is encouraged when allowed by national/regional regulations
- **patient advocacy organisations (PAOs - see more information below and refer to the INSERM contact point) – TBC**


Other eligibility rules:

- Min. of 4 eligible partners from 4 different countries
- Max. of 6 partners per project that can be extended to 8 if underrepresented countries (CZ,EE,HU, LT, PL, SK, TK) participate
- The coordinator must be always eligible
- Associated partners from countries NOT participating in the call may be part of the project if they participate with their own funding, their number is limited to 2 (check details in the call rules)
- Timetable:
 - Call opens on 14/12/2018
 - Pre-proposal submission closes on 14/02/2019
 - Invitation of selected project to submit full proposal – early May 2019
 - Submission of full proposals 11/06/2019
 - Final results – October 2019

<http://www.ejprarediseases.org/index.php/documents/>

Applications x pixel, l'intranet HCL x Documents - EJP RD x

www.ejprarediseases.org/index.php/documents/

 **EJP RD**
European Joint Programme on Rare Diseases

Home About Funding v Contact

DOCUMENTS

EJP RD Joint Transnational Call 2019 Documents:

[EJPRD JTC 2019 - Call text](#)

[EJPRD JTC 2019 - Guidelines for applicants-update 07_01_19](#)

[Pre-Proposal_Form_EJPRD_JTC2019 VF](#)

Specific documents for eligibility pre-check available to download:

[FRRB eligibility pre-check form](#)

[FRRB eligibility pre-check budget tool](#)

FR 11:19
14/01/2019



EJP RD JTC 2019: Guidelines for applicants

European Joint Programme on Rare Diseases (EJP RD)

Call for Proposals 2019

"Transnational research projects to accelerate diagnosis and/or explore disease progression and mechanisms of rare diseases"

Guidelines for applicants

Submission deadline for pre-proposals: February 15, 2019

Submission deadline for full proposals: June 11, 2019

The links to pre-proposal template, electronic proposal submission, guidelines for applicants can be found at the EJP RD website: www.ejprarediseases.org



EJP RD JTC 2019: Guidelines for applicants

FRANCE, ANR

Country	France
Funding organisation	French National Research Agency (Agence nationale de la recherche –ANR-) http://www.agence-nationale-recherche.fr
National contact person	Health & Biology Department Agence Nationale de la Recherche –ANR 50 avenue Daumesnil - 75012 Paris, France Florence Guillot Email: E-RareCalls@agencerecherche.fr Phone: (33) (0) 1 78 09 80 01
Funding commitment	3 M€
Anticipated number of fundable research partners	10 research partners The ANR has a maximum funding per coordinator/partner for this call: Each research team can be funded with a maximum amount of 300 000 € for a French coordinator and 250 000 € for a French partner. There is a minimum amount per partner: 15 000 €
Eligibility of project duration	2-3 years
Eligibility of a partner as a beneficiary institution	Eligible institutions: - Public research institutes such as EPST, EPIC, universities, university hospitals, non-university research institutes (max. rate of support: 100% of marginal costs) - Enterprises: large & SMEs (max. rate of support: 45% of total costs for SMEs & 30% for larger companies) Additional eligibility criteria: - The coordinator (if from a French institution) must belong to a public research organisation. - ANR will avoid double funding and will not finance projects or part of projects that have been funded through other calls. ANR will cross-check the proposals submitted to ANR through the national and international calls for possible demands of double funding.
Eligibility of costs, types and their caps	Personnel costs for temporary contracts; small equipment; consumables and animal costs; travel; and sub-contracting, if necessary to carry out the proposed activities (sub-contracting costs of max 50% of requested budget per partner). Please note that at ANR « overheads » means « frais généraux de gestion – frais de structure », and 8% of the total eligible costs must be applied if the partner belongs to a public research organisation, or 68% of the total personnel costs



EJP RD JTC 2019: Guidelines for applicants

	and 7% of other costs if a partner belongs to another category (cf "Règlement financier ANR – section 4.2.3.5)
Submission of the proposal at the national level	No
Submission of other information at the national level	Yes. Contacting the ANR national contact point is mandatory.
Submission of financial and scientific reports at the national level	Yes. Financial reporting is submitted to ANR financial modalities and must be followed according to the contract that will be signed with the future beneficiaries. Scientific reports: individual scientific reports are not required. However, French partners should contribute to the central report to be submitted by the coordinator of the project to EJP RD. This report will be the basis for validation of yearly advancements of the project by ANR.
Further guidance	Plan d'Action 2018: http://www.agence-nationale-recherche.fr/PA2018#documents Règlement financier: http://www.agence-nationale-recherche.fr/fileadmin/documents/2017/ANR-Reglement-financier-2017.pdf