ERDERA
European Rare Diseases
Research Alliance

Call for Proposals 2025 pre-announcement

"Pre-clinical therapy studies for rare diseases using small molecules and biologicals – development and validation"

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 **European Rare Diseases Research Alliance (ERDERA)** 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). These actions follow the five Joint Transnational Calls for rare diseases research projects launched previously by the European Joint Programme on Rare Diseases (EJP RD) since 2019.
A number of national and regional funding organisations intend to participate in the **ERDERA Joint Transnational Call (JTC) 2025** to fund research projects on rare diseases. The call is supposed to open 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
- Public Service of Wallonia (SPW), Belgium, Wallonie
- Bulgarian National Science Fund (BNSF), Bulgaria
- Canadian Institutes of Health Research – Institute of Genetics (CIHR-IG), Canada
- The Research and Innovation Foundation (RIF), Cyprus
- Ministry of Health (MZCR) / Czech Health Research Council (AZVCR), Czech Republic
- Innovation Fund Denmark (IFD), Denmark
- Estonian Research Council (ETAG), Estonia
- French National Research Agency (ANR), France
- Foundation For Rare Diseases (FFRD), France
- Federal Ministry of Education and Research (BMBF), Germany

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- German Research Foundation (DFG), Germany
- National Research, Development and Innovation Office (NKFIH), Hungary
- The Icelandic Centre for Research (RANNIS), Iceland
- 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 (MUR), Italy
- Fondazione Telethon (FTELE), Italy
- Regional Foundation for Biomedical Research (FRRB), Lombardy (Italy)
- Tuscany Region (RT/TuscReg), Tuscany (Italy)
- Latvian Council of Science (LZP), Latvia
- Research Council of Lithuania (LMT), Lithuania
- National Research Fund (FNR), Luxembourg
- The Research Council of Norway (RCN), Norway
- National Centre for Research and Development (NCBR), Poland
- The Foundation for Science and Technology (FCT), Portugal
- Slovak Academy of Sciences (SAS), Slovakia
- National Institute of Health Carlos III (ISCIII), Spain
- Vinnova, Sweden
- Swiss National Science Foundation (SNSF), Switzerland
- Netherlands Organisation for Health Research and Development (ZonMw), The Netherlands
- The Scientific and Technological Research Council of Türkiye (TUBITAK), Türkiye

The final decision of all funding agencies to participate in the call is still pending and can be subject to change.

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 the expected impact being future use of the results to benefit patients.

Projects will focus on **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 relevant criteria and commonalities if this leverages added value in sharing resources or expertise.

Topic: “Pre-clinical therapy studies for rare diseases using small molecules and biologicals – development and validation”

Topics List

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Research studies on therapies using small molecules, small non-coding chemically synthesized nucleic acid-based therapies, repurposed drugs or biologicals (e.g., antibodies or proteins such as enzymes, immune modulators or growth factors etc.). Proposals must cover **at least one** of the following areas**:**

1. development of novel therapies in a preclinical setting through cell, organoid and animal model studies, molecule screening or use of *in silico* or artificial intelligence models
2. development of predictive and pharmacodynamics biomarkers correlated to the efficiency of the therapy in a preclinical setting that could serve as surrogate endpoints
3. replication of pre-clinical studies in an independent lab to increase validity of exploratory findings
4. pre-clinical proof of concept studies for evidence of pharmacological activity *in vitro* and *in vivo*, pharmaco-kinetics and pharmaco-dynamics of the drug and first toxicology and safety data
5. studies to support readiness for initiating clinical trial authorization conforming to regulatory requirements

Translatability into humans should be the key focus of the project, and applicants should demonstrate access to relevant scientific or regulatory expertise (e.g., through innovation task forces or competent national authorities).

Excluded Approaches and Topics

The following approaches and topics are excluded from the scope of the JTC 2025:

- ATMP therapies (gene therapy medicinal product (including mRNA-based therapies), somatic cell therapy medicinal product, tissue engineered product, according to EMA definition).
- Development of new cell/organoid/animal models, which should already be established.
- Set-up or extension of natural history studies / patient registries.
- Interventional clinical trials to prove efficacy of drugs/treatments/surgical procedures/medical procedures. This includes studies comparing efficacy, e.g., two surgical techniques or therapies, and projects whose main objective is the implementation of a clinical phase IV pharmacovigilance study.
- Projects focusing only on rare neurodegenerative diseases that are within the focus of the Joint Programming Initiative on Neurodegenerative Disease Research (JPND). These are: Alzheimer's disease and other dementias; Parkinson's disease (PD) and PD-related disorders; Prion diseases; Motor Neuron Diseases; Huntington's disease; Spinal Muscular Atrophy and dominant forms of Spinocerebellar Ataxia. Interested researchers should refer to the relevant JPND calls. **However, childhood dementias/neurodegenerative diseases are eligible.**
- Rare infectious diseases, rare cancers and rare adverse drug events in treatments of common diseases. **Rare diseases with a predisposition to cancer are eligible.**

Funding and Categories of Partners

The maximum duration of the project is three years.

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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).

Consortium Makeup

Limit number of partners

Only transnational projects will be funded. Each consortium submitting a proposal must involve **four to six eligible principal investigator partners (referred to as partners below)** from **at least four different participating countries** (see list in section 2). **One of these four to six partners must be an Early Career Researcher (ECR)**. In specific cases this number of consortium partners can be increased to eight partners (see below). No more than two eligible partners from the same country can be present in each consortium. Patient advocacy organisations (PAOs) requesting funding do not count toward the total.

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

- The inclusion of partners from participating countries usually underrepresented (UR) in projects (UR: Czech Republic, Estonia, Latvia, Lithuania, Slovakia, Türkiye, *additional countries tbd*), OR
- The inclusion of an additional ECR as full partner (see section 5.6).

Number of research partners requesting national/regional funding	Inclusion of UR partner	Inclusion of ECR partner
4	Not mandatory	1 mandatory
5		
6		
7 (only possible with inclusion of one partner from UR countries or additional ECR)	1	1 additional ECR partner
8 (only possible with inclusion of two partners either from UR countries or additional ECRs)	1-2	1-2 additional ECR partners

What is a partner? a collaborator? a sub-contractor?

To be **considered as an eligible partner**, a group must contribute substantially to at least one of the project's work packages. If the only role of a group is to provide patient access, data or samples for the study, they will not be considered as partners of the consortium, but can be included otherwise, via cooperation agreements (as collaborators) or subcontracting.

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Consortia may include **collaborators** that secure their own funding. Collaborators cannot be work package leaders, and their contribution to the consortium must be described. As they do not receive funding as part of this call, they do not count toward the limit of 8 partners requesting research funding (nor is there a limit of collaborators per country, as long as their participation is justified). Collaborators must supply a letter of intent and a CV as well as be entered in the online submission system.

If necessary, to implement the research activity, consortia may also include **sub-contractors according to country/regional regulations**. Sub-contractors may cover only a limited part of the research activity, and their contribution to the consortium must be described. They do not count toward the limit of 8 partners requesting research funding (nor is there a limit of subcontractors per country, as long as their participation is justified and if subcontracting is possible according to national/regional funding rules).

Patient Advocacy Organisations and Patient Involvement/Partnership

Consortia are expected to include and actively engage patient partners (patients/caregivers/family members) and/or patient advocacy organisations (PAOs) from the start when preparing their proposals.

Funding for PAOs through the central funding mechanism administered by ZonMw is limited to a total of 25,000 € over 3 years and per project regardless of the number of participating PAOs. In addition, PAOs can also be involved through national/regional funding or subcontracting depending on the proposed tasks and national/regional funding rules.

Early Career Researchers

At least one Early Career Researcher (ECR) must join a consortium as a full research partner and is therefore subject to the same eligibility criteria as other partners. ECRs must demonstrate independence and scientific excellence, and should be clearly identified in the proposal and their CV.

In general, ECRs can either be PhD holders or medical doctors.

PhD holders

Scientist who has received their PhD no more than seven years prior to the application deadline.

Medical doctors

Physician who has completed specialist medical training no more than seven years prior to the application deadline. For physicians with a PhD, the date of the completed specialist medical training remains the relevant date.

Extensions to this period are allowed in case of reasonably justified career breaks: absence for parental leave, family care leave, long-term sickness leave, and compulsory military service.

Please note that national/regional eligibility criteria, definitions and time limits might differ.

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 10th, 2024**.

Call Timeline

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ERDERA Joint Transnational Call 2025 – Preliminary announcement

10th December 2024	Launch of the call
17th December 2024	Information webinar for potential applicants
13th February 2025	Pre-proposal submission deadline
Early May 2025	Invitation to full proposal
6th May 2025	Information webinar for applicants invited to submit a full proposal
9th July 2025	Full proposal submission deadline
December 2025	Notification of funding decision

For general questions regarding the joint call please contact the Joint Call Secretariat

DLR Projektträger (DLR-PT, Germany)

Dr. Katarzyna Saedler
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For questions regarding national eligibility criteria and requirements please contact the national contact person listed below

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NATIONAL CONTACT POINTS

Country/ Region	Funding Organisation	Contact Details
Austria	Austrian Science Fund (FWF)	Dr. Doris Lucyshyn Doris.lucyshyn@fwf.ac.at DI Anita Stürtz Anita.stuertz@fwf.ac.at
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