ERDERA

European Rare Diseases
Research Alliance

European Rare Diseases Research Alliance

Daria JULKOWSKA
ERDERA coordinator
INSERM, France

Joint Transnational Call 2026 Webinar
16 of December 2025, Online

Co-funded by the European Union

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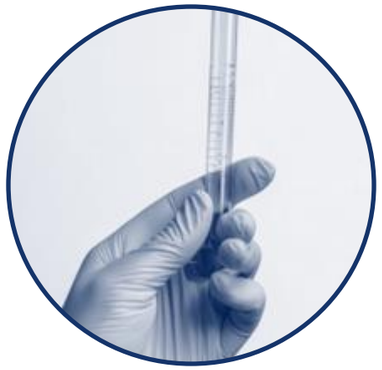
Improving the health and well-being of 30 million people living with a rare disease by making Europe a world-leader in RD research and innovation.



Diagnosis established or enrolment in systematic research in average within 6 months after coming to medical attention

New effective therapies approved in Europe and beyond, the majority of which addressing diseases without approved options

Better understanding of the impact of rare diseases on patients, families and society to improve quality of life

ERDERA
European Rare Diseases Research Alliance

ERDERA: Our Global Scale & Investment

Diverse Partnership Network

Our mission is powered by a broad network of nearly **180 organizations from 37 countries**, including:

- Funders
- Research Institutions
- Hospitals
- Research Infrastructures
- Patient Organizations
- Industry Partners

Extensive Global Reach

ERDERA spans **25 European Union countries** (except Croatia and Malta) and **numerous associated and non-EU countries**, fostering international cooperation (Australia, Canada, Iceland, Israel, Georgia, Morocco, New Zealand, Norway, Serbia, Switzerland, Türkiye, United Kingdom)

Substantial Financial Commitment

A significant financial commitment underpins our ambitious goals, with a total budget of approximately **€380 million**:

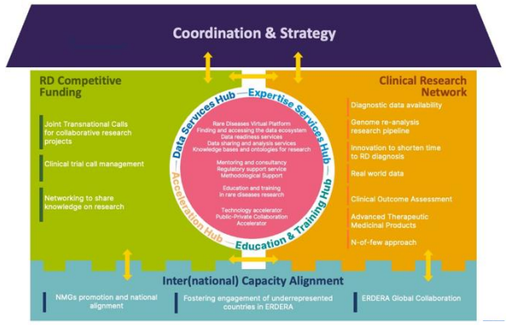
- **€150 million** investment from the European Commission
- Over €230 million contributed by participating countries and institutions
- **Every partner contributes** to our collective actions

ERDERA brings together diverse expertise and significant resources to advance its mission, demonstrating a strong commitment to global collaboration.

NMGs promotion and national alignment

Fostering engagement of underrepresented countries in ERDERA

ERDERA Global Collaboration



ERDERA's research & innovation support cycle

Competitive and In-house Research & Innovation

ERDERA Research Funding

Mentoring & Education

National capacity & growth

Tools & Services to Enhance Outputs

Exploratory Due Dilligence

Public-private Accelerator

Clinical Research Network

ERDERA European Rare Diseases Research Alliance

Clinical Research Network

DIAGNOSIS

- Diagnostic data availability
- Genome re-analysis research pipeline
- Innovation to shorten time to RD diagnosis

CLINICAL TRIAL READINESS

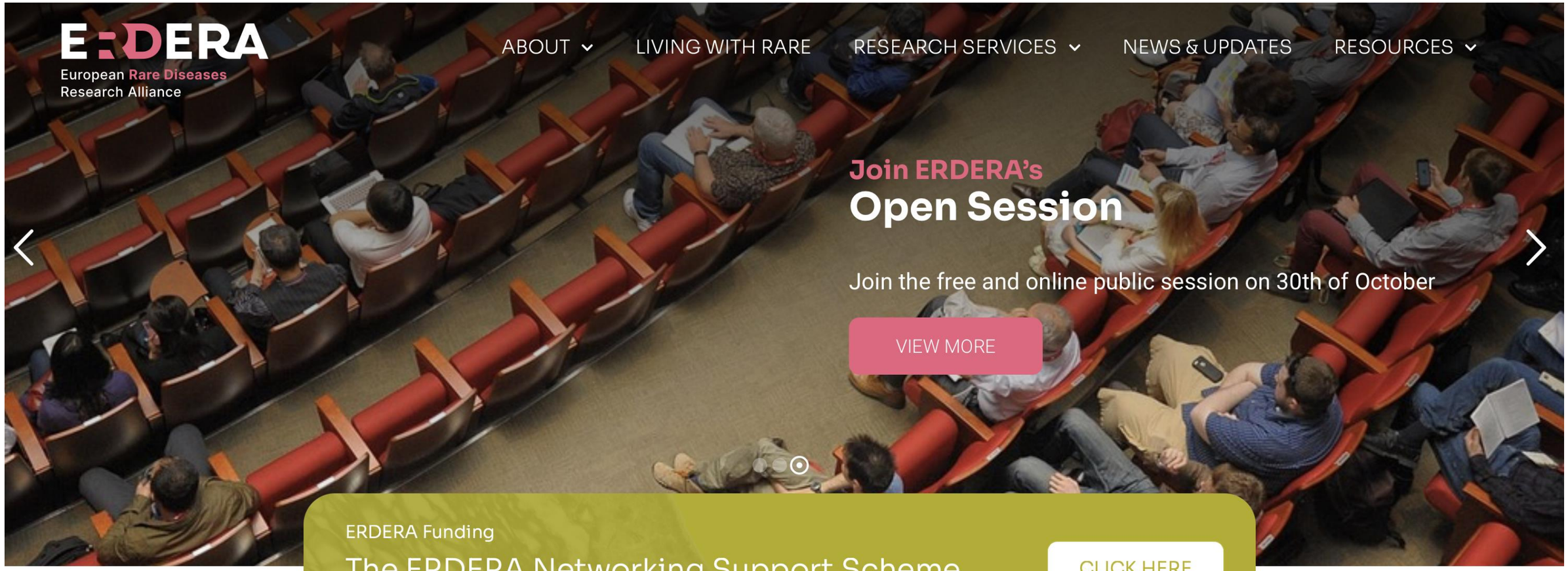
- Real world data
- Clinical Outcome Assessment

THERAPY DEVELOPMENT

- Advanced Therapeutic Medicinal Products
- N-of-few approaches

ERDERA in house research

ERDERA European Rare Diseases Research Alliance



www.erdera.org

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Thank you!

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ERDERA | Funding

Information Webinar

ERDERA Joint Transnational Call 2026

Resolving unsolved cases in rare genetic and non-genetic diseases

Florence Guillot, ANR
16 December 2025

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Agenda

15.00-15.10	**Welcome and Introduction to ERDERA**	**(Daria Julkowska)**
15.10-15.30	**JTC 2026**	**(Florence Guillot)**
15.30-15.40	**Patient engagement in research**	**(Roseline Favresse, Avril Kennan, Laura Benkemoun)**
15.40-15.50	**ERDERA expertise support services**	**(Rosan Kreeftmeijer-Vegter - EATRIS)**
15.50-16.00	**Q&A**	

Aim of the call - Diagnosis

- Challenge: Accurately diagnosing RDs

  - 50% of individuals with a suspected rare genetic condition remaining undiagnosed or misdiagnosed
  - RDs of non-genetic origin - estimated to account for about 10% of all RD cases - remain an under-investigated area
  - 5 years to establish an accurate diagnosis for people living with a RD

- Objective:

  Solve **Undiagnosed Rare Genetic and Non-Genetic diseases**

  Address **complex, multifactorial diseases**

  by identifying causative variants in patients with no molecular diagnosis after prior genetic or genomic testing and providing diagnostic clarity for conditions of unknown or mixed pathogenesis.

Call topic: Resolving unsolved cases in rare genetic and non-genetic diseases

At least one of these areas:

- Functional validation to classify variants of uncertain significance (VUS) and increase the diversity of functional genomics research, or validation of candidate VUS to improve outcomes for a broader range of patients using in silico, in vitro or animal model systems (e.g. CRISPR modified cells, iPSCs, organoids, etc.);
- Use of multi-omics or integrative methods (e.g. transcriptomics, epigenomics, etc.) to resolve ambiguous or complex variants;
- New tools/methodologies not yet validated in clinical settings, including biostatistics, advanced bioinformatics, and mathematics approaches (e.g. variant effect predictors, Artificial Intelligence (AI)-based annotation platforms, etc.);
- Systems biology and disease mechanism modelling;
- Integration of clinical, environmental, lifestyle, and sensor-derived data;
- Development of knowledge graphs or disease maps to link phenotypic and mechanistic insights;
- Use of advanced AI and modelling tools (graph ML, probabilistic causal models).

Excluded Diseases, Approaches and Topics

- Pre-clinical therapy development studies as covered in ERDERA JTC2025 topic;
- 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.**

Frequently asked questions

- Is disease xyz considered a rare disease?

Refer to definition „disease affecting not more than five in 10.000 persons in the European Community, EC associated states, and Canada”,

consult Orphanet or scientific literature for prevalence data : ORPHAcode: mandatory field.

*Use **ORPHAcode 616874** (Rare disorder without a determined diagnosis after full investigation) for undiagnosed cases.*

- Does the proposal necessarily need to address variant identification ? or can it address only identification of predictive biomarkers for diseases with yet unpredictable outcomes (with no search for genetic variants) ?

- Would the use of AI be considered to predict diseases associated with possible undiagnosed cases in this call?
- Can the proposal be submitted regarding one / several defined variants in novel genes suspected of causing immune dysregulation or immunodeficiency.
  - Yes to all

General considerations 1

- Proposals should integrate sex and gender considerations, underrepresented populations, or underrepresented patient sub-groups;
- Appropriate bioinformatics and statistical methods;
- Leveraging existing infrastructures such as biobanks and model repositories (e.g., EuroBioBank, RD-Connect) and ensuring compliance with obligations to make generated resources publicly available, including depositing new mouse models or cellular lines in recognized repositories in accordance with FAIR principles;
- Generative AI (GenAI) approaches may be used only when their application is clearly justified, ethically sound, and demonstrably offers advantages over conventional methods. Projects proposing GenAI must ensure full compliance with relevant EU legislation and ethical frameworks, including the AI Act, the GDPR, and the JTC 2026 Guidelines

General considerations – Data Services Hub (DSH)

- Data generated or newly collected for the project must be made ready for reuse in accordance with the Findable, Accessible, Interoperable and Reusable (FAIR) Guiding Principles. Applying ERDERA's FAIR data stewardship services will contribute to a new data resource to the ERDERA Data Hub (https://erdera.org/data-hub/).
- The Data Hub and the associated Virtual Platform (VP; https://erdera.org/erdera-virtual-platform/) are collective achievements of the ERDERA partnership that enable automated applications across multiple data and knowledge sources

- **Contact: jtc-requests@erdera.org**

General considerations – CRN Diagnostics

- The genomic and other -omics data from participants and related family members generated by the funded projects should be shared with the ERDERA Diagnostic Research Workstream (https://erdera.org/clinical-research/erdera-diagnostics/) at most one year after being generated if the index case remains genetically undiagnosed.

Contact: jtc-requests@erdera.org

Eligible countries/regions and budget

- **31 funding agencies from 26 EU, EU associated countries & Canada, co-funded by European commission**
- **Participating countries:** Austria, Belgium, Bulgaria, Canada, Cyprus*, Czech Republic, Denmark, Estonia, France, Germany, Hungary, Ireland, Israel, Italy, Latvia, Lithuania, Luxembourg, Norway, Poland, Portugal, Slovakia, Slovenia, Spain, Sweden, The Netherlands, Türkiye and Malta as collaborator
- **Each funder funds only their respective national/regional teams → check guidelines!! → contact your national/regional contact point!!**
- Partners from other countries (e.g. AU, BR, CH, UK, …) can only participate as collaborators with own funding
- **3 years projects**
- **Overall budget: 31 Mio €**
- **Expected number of funded projects: ~20**
- Usual success rate: 10-15 %

Consortium partners and composition (1)

- **Categories of Partners** (depending on national/regional regulations)
  - Academia (research teams working in universities, other higher education institutions or research institutes),
  - Clinical/public health sector (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**
  - **four to six** eligible principal investigator **partners** from at least **four different participating countries**
  - **The number of partners can be increased to 8 in two cases:**
    - The inclusion of partners from participating countries usually underrepresented in projects (UR: Estonia, Latvia, Lithuania, Slovakia, Türkiye), OR
    - The inclusion of an Early Career Researcher as full partner
  - Patient advocacy organisations do not count toward the total number of partners in the consortium
  - No more than 2 eligible partners from the same country in a consortium (further national/regional limits may apply)

Consortium partners and composition (2)

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

  - **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.

  - **Collaborator:** research groups that secure their own funding, can come from a non-participating country. 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 in the online submission system.

  - **Sub-contractor**: 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

- 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.

- The consortia should clearly **describe the role and responsibilities** of the patient partners and PAOs, how they will operate, at what levels and stages of the research, and provide justifications for allocated resources in a patient involvement plan.

- PAOs representative of rare disease patients within a Member State or throughout the EU/EEA can be funded through **central funding mechanism**, max 25.000 € per project – please check guidelines!
- Patient partners may also participate through national or regional funding schemes, or via subcontracting, depending on national or regional rules

Frequently asked questions

- Each funder will finance the teams from its own country/region, therefore each partner **must fulfill** the financial rules of its funder and respect the maximum amount allowed per project or research team;
- Some funders request a parallel submission nationally/regionally, please check carefully the guidelines;
- Collaborators: experiments in-kind (letter of content with description of the tasks, justification of the costs with grant number etc…);
- No budget transfer is allowed within the research partners/collaborators of the consortium

Application Timeline

Call opens
December 10, 2025

Informational webinar
December 16, 2025

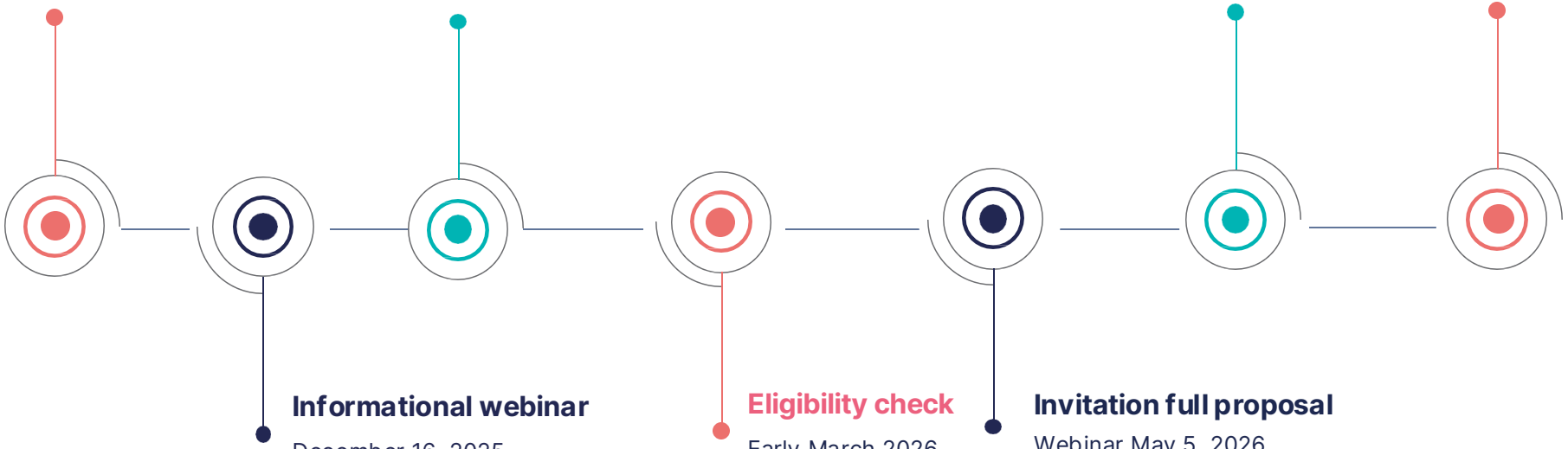
Pre-proposal submission
February 12, 2026

Eligibility check
Early March 2026

Invitation full proposal
Webinar May 5, 2026

Full proposal submission
July 8, 2026

Decision
Dec 2026



Registration and submission

- One joint pre-proposal (in English) to be submitted by coordinator to JCS via the electronic submission system: https://funding.erdera.org **no later than February 12th, 2026 at 2:00 p.m. (CET)**
- **Call documents available for download at https://erdera.org/funding/**
- **PRE-PROPOSAL SUBMISSION FORM PREVIEW ONLY PROVIDES INFORMATION ABOUT THE SUBMISSION PLATFORM. IT IS NOT INTENDED TO BE FILLED OUT AND UPLOADED. ONLY PROPOSALS THAT HAVE BEEN ENTERED ELECTRONICALLY AT FUNDING.ERDERA.ORG WILL BE EVALUATED!**
- **CHECK PREVIEW, PREPARE NECESSARY DATA, ONLY PLAIN TEXT CAN BE ENTERED FOR DESCRIPTIONS.**
- **CV TO BE FILLED OUT ONLINE FOR PARTNERS ONLY**
- **Documents to upload in the platform**
  - Diagram of the work plan (Timeline, workflow and interconnections of work packages (Gantt chart, Pert or similar)
  - Diagrams, figures, tables etc. to support the work plan description
  - List of references
  - Date and signature page of all project partners asking for funding
  - Collaborators: Letter of intent for collaborators
  - PAOs: Declaration of honor and *Minimis* Statement
- ***Eligibility pre-check / parallel submission necessary for some funding agencies***

Evaluation

- **Scientific Evaluation Committee (SEC)** consisting of internationally recognised, independent, scientific experts from the fields of biomedical science (e.g. basic research, diagnostic research, clinical research, animal/cell model expertise) plus experts in patient engagement (full proposal only)
- **Evaluation criteria** according to Horizon Europe for **pre-proposals:**
  - **Excellence**
    - Clarity and pertinence of the objectives;
    - Credibility of the proposed approach and methodology;
    - Soundness of the concept;
    - Innovative potential:
    - Competence of participating research partners
  - **Impact**
    - Potential and readiness of the expected results to be translatable for future clinical applications
    - Benefit to patients, their families, and carers;
    - Inclusion of Early Career Researchers;
    - Added value of transnational collaboration;
    - Involvement of industry
  - **Quality and efficiency of the implementation**
    - Feasibility of the project
    - Complementarity of the participants within the consortium, including the integration of PAOs or patient partners;
- **Scoring** – see call text
- **Full proposal**: additional criteria, independent ethical evaluation

Frequently asked questions

- Is there a partnering platform for this call?
  - Unfortunately, there is no partnering platform available for this call. To find RD experts: https://funded-projects.ejprarediseases.org/dashboard
  - Orphanet: https://www.orpha.net/en/institutions

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Thank you!

To register to our newsletter:
https://erdera.org/#keepconected

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European Rare Diseases
Research Alliance

Patient partnerships

Why and how to involve patients in research from the start?

Roseline Favresse, EURORDIS-Rare Diseases Europe
On behalf of the ERDERA PPIE Group

JTC2026 webinar
16 December 2025

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Patient partners as JTC partners

- Consortia should **include and actively engage** at least one patient partner (i.e. a patient representative from an organized group (preferably from a patient advocacy organization)
- **From the start** when preparing their proposals.
- Funding possible through the **central funding mechanism** (managed by ZonMw, EC budget). Budget **per project** limited to a total of **€25,000 over 3 years**
- Alternatively, patient partners may participate through national or regional funding schemes, or via subcontracting → contact your national/regional contact point for further information.
- **Any funding claim should be consistent with the tasks and roles of the patient partnering organisation**

Which role?

- The consortium should **clearly describe the role and responsibilities of the patient partners**, how they will operate, at what levels and stages of the research, and provide justifications for allocated resources in a **patient involvement plan**.
- It is expected that patient partners are involved in **advisory/steering/governance group**.
- It is expected that patient partners are involved **throughout the research lifecycle where appropriate**, and **advise on issues** such as:
  - identifying and prioritising the research question,
  - planning,
  - design,
  - conduct,
  - analysis,
  - oversight and governance, and
  - dissemination.

What is a Patient Advocacy Organisation?

Definition of rare disease patient advocacy organisations:

- Patient advocacy organisations are defined as not-for-profit organisations, which are patient focused, and where patients and/or carers and/or family members of patients represent a majority of members in governing bodies. These are:
  - Umbrella organisations (e.g. representing either European organisations and/or national umbrella organisations for rare diseases).
  - European rare disease specific organisations (i.e. representing national organisations or individual patients on rare diseases) and
  - National rare disease specific organisations.

How do I find a Patient partner / Patient advocacy organisation (PAO)?

Existing databases and directories

- EURORDIS – Rare Diseases Europe
- ORPHANET – Directory of patients' organisations
- Rare Diseases National Alliances
- European Patient Advocacy Groups (ePAGs)

What if there are several patient organisations in place for the same disease?

→ Check if a European federation/association exists and is active for the disease

→ Try to identify the patient organisation that is more involved in research activities

What if there is no patient organisation for the disease we target?

→ Check with the relevant umbrella organisations (there may be an association in a different country)

→ There is still an opportunity to involve an umbrella organisation that may be active in the group of diseases you target

→ Foresee the involvement of individual patients in activities at some point of time if there is no structured organisation in place to advise/co-design

PPIE, what it is?

Patient and Public Involvement and Engagement: working **with** patients and the public to shape research and engage with it.

Patient and public involvement and engagement (PPIE) describes the **different ways in which members of the public can inform and shape research.**

"PPIE is different to research participation where members of the public can directly take part in a study, for example by being given a new treatment as part of a clinical trial." – University of Birmingham

*"Patient and public involvement (PPI) entails **research being carried out 'with' or 'by'** members of the public, rather than 'to', 'about' or 'for' them"*- National Institute of Health Research

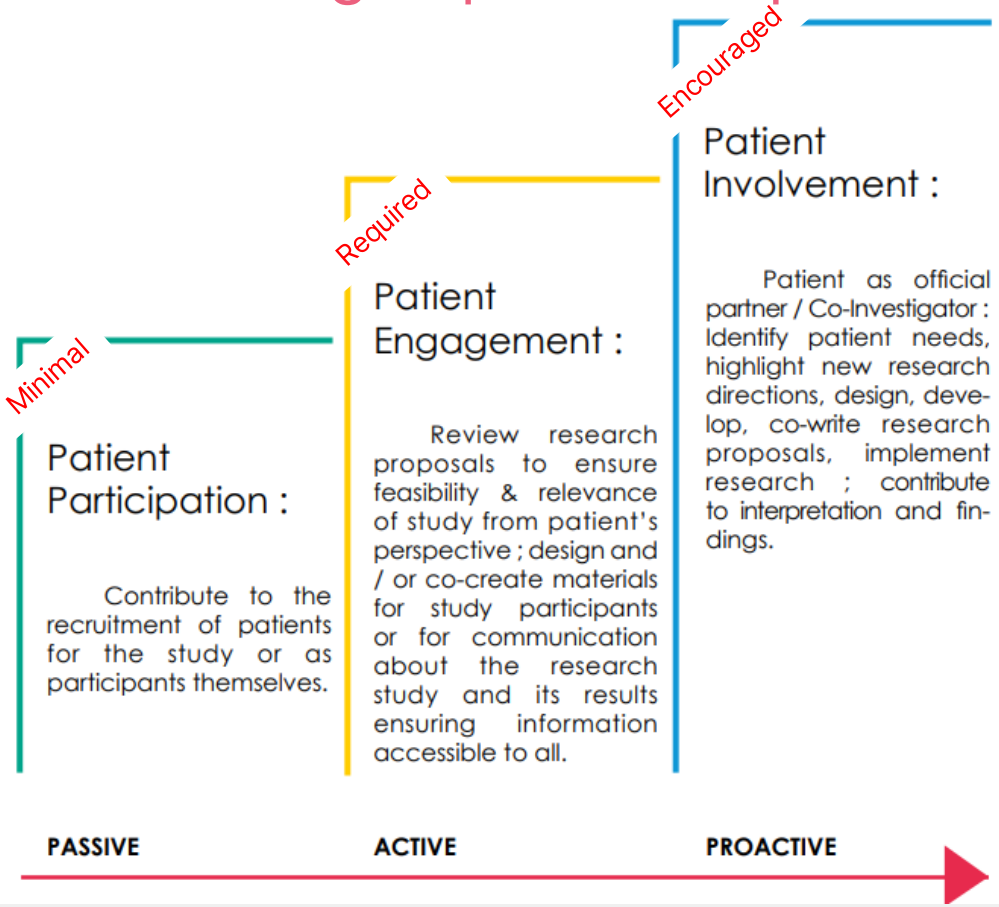
PPIE is for **everyone.**

What is patient partnership? The PENREP Short Guide

- Defines what patient partnerships are
- Defines the benefits of involving patients
- Provides examples of concrete patient partnerships
- Highlights the common pitfalls that could be avoided
- Provides an indicative checklist for self-evaluation
- Embeds testimonies from successful applicants

A continuum of active and meaningful partnership

Depending on the project and its state of development, all these options may happen concomitantly or iteratively/progressively



Patient partnerships' benefits

STRONGER FUNDING APPLICATIONS

GREATER RELEVANCE

MOTIVATION & FOCUS

EXPANDED OUTREACH & IMPROVED COMMUNICATION

NEW IDEAS

GREATER IMPACT

Succeeding in partnerships between patient organisations & researchers

- What should a good partnership look like?
- Understanding the charity perspective
- Understanding the researcher perspective
  - Recommendations for charities & other civil society organisations
  - Recommendations for researchers
  - Recommendations for universities and other research institutions
  - Recommendations for research funders

https://hrci.ie/hrci-ppi-ignite-network-charities-researchers-partnering-guide/

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Avoiding common pitfalls (examples)

" Patient organisations will recruit patients as donors for the biobank . "

Not enough explanation is given as to how this will be achieved. Who ? How ? When ? Was the patient organisation involved in developing the recruitment strategy ?
If involvement / engagement activities are not planned, please provide an explanation as to why it was not possible in this project.

" The applicants are in contact with patients and patient organisations so patients will be engaged / involved throughout the research project "

" Patient organisations will be responsible for disseminating the research results to their communities "

Any specific roles and responsibilities need to be discussed and agreed between the researchers and the patient organisations (or patients) before submitting the proposal and need to be detailed in the proposal.

Generic statements are not useful to evaluators and need to be expanded to include the descriptions of the responsibilities of the different partners.

Checklist for self-evaluation of applicants (extract)

- ☐ *Have discussions between researchers and patient representatives taken place before identifying the research questions and writing the proposal ?*
- ☐ *Have you described how the patients/patient representatives were identified and selected ?*
- ☐ *Has the input of patients/patient representatives been integrated in the development of the proposed research project ? Have you described what changed / improved as a result of this input ?*
- ☐ *Have clear roles and responsibilities been assigned to the patients / patient representatives in the project ?*
- ☐ *Have the Patient Partnership activities been clearly explained (who, what and when) ?*
- ☐ *Have the available resources of respective partners been maximised to the benefit of the research project (e.g. registries, know-how, networks, communication channels) ?*
- ☐ *Have the approaches through which the patients / patient representatives will be engaged / involved / participate in the project been described (e.g. focus groups, interviews, surveys etc.) ?*
- ☐ *Has a process been included to ensure two-way communication between the partners throughout the life of the project ?*

European **Rare Diseases** Research Alliance

Thank you!

Roseline Favresse - roseline.favresse@eurordis.org

On behalf of all ERDERA PPIE Group

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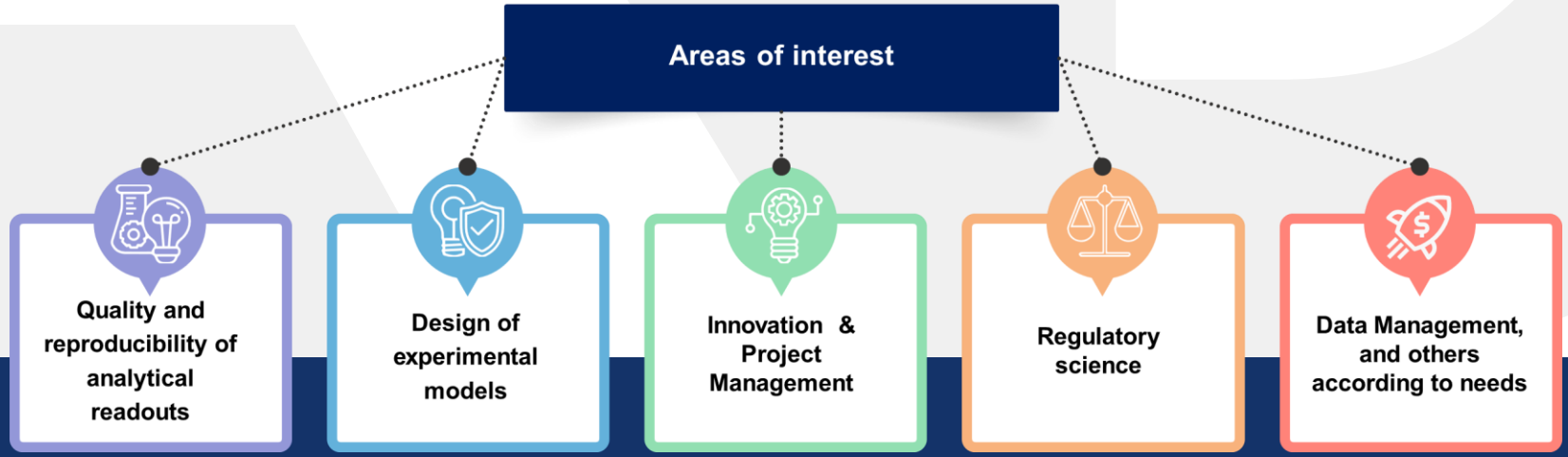
ERDERA Mentoring service

Agustín Arasanz
(VHIR/ EATRIS)

ERDERA European Rare Diseases Research Alliance

Execution of the ERDERA Mentoring service

- **To Whom**: Shortlisted JTC's proposals and also projects from other funding schemes
- **Format**: Webinar and 1-on1 meetings with expert mentors who provide advice on specific areas
- **Cost**: Free of charge, full confidentiality (signed Letter of engagement/CDA)
- **When**: During 2nd stage to prepare the final proposal , through full project lifetime



Mentoring organization

- **Kick-off Webinar to all shortlisted applicants (1.5h)**
  Covers common challenges — no confidential information is shared.

- **Mentoring Request Form**
  Captures essential project information, LoE and supports triaging (matching to right experts)

- **Onboarding Calls (1h)**
  - Identify the specific needs of each individual Project (from Pis or reviewers)
  - Provide general guidance on biomarker development, regulatory aspects, data management, and intellectual property (IP).

- **Follow up by email**
  - Share report and Resources
  - Possibility for further exchange via email

- **Additional expert meeting (phase 2) or expert service upon request**

Funded by the European Union
GA n°825575

ERDERA Timeline – JTC application & Mentoring

Call opens

December 10, 2025

Informational webinar

December 16, 2025

Pre-proposal submission

February 12, 2026

Shortlisting

April, 2026

Kick off Webinar

May 5, 2026

1-on-1 mentoring meetings

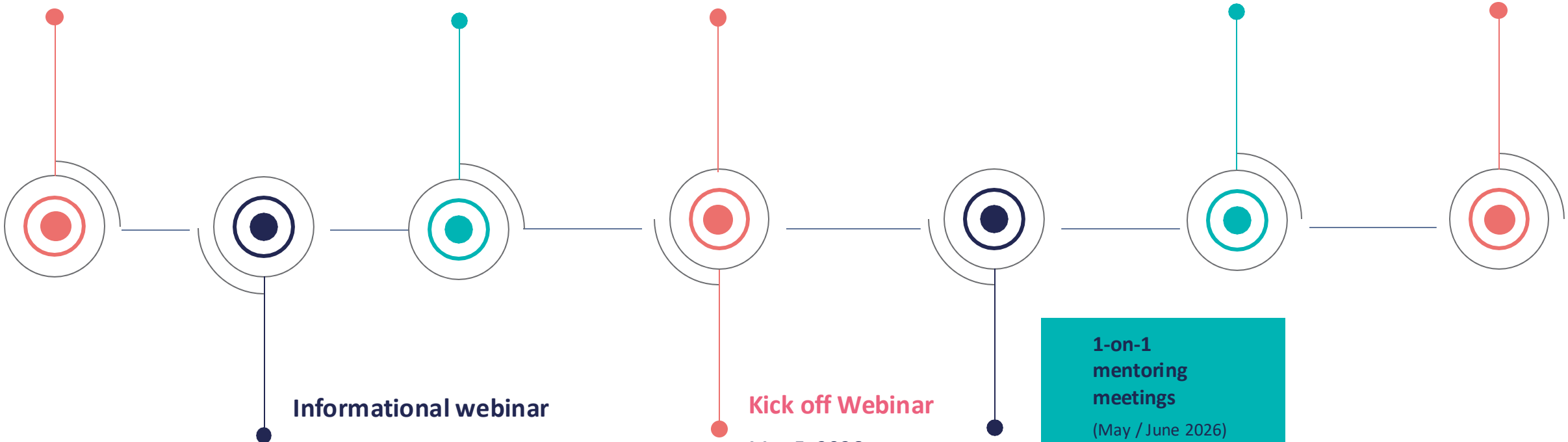
(May / June 2026)

Full proposal submission

July 8, 2026

Decision

Dec 2026



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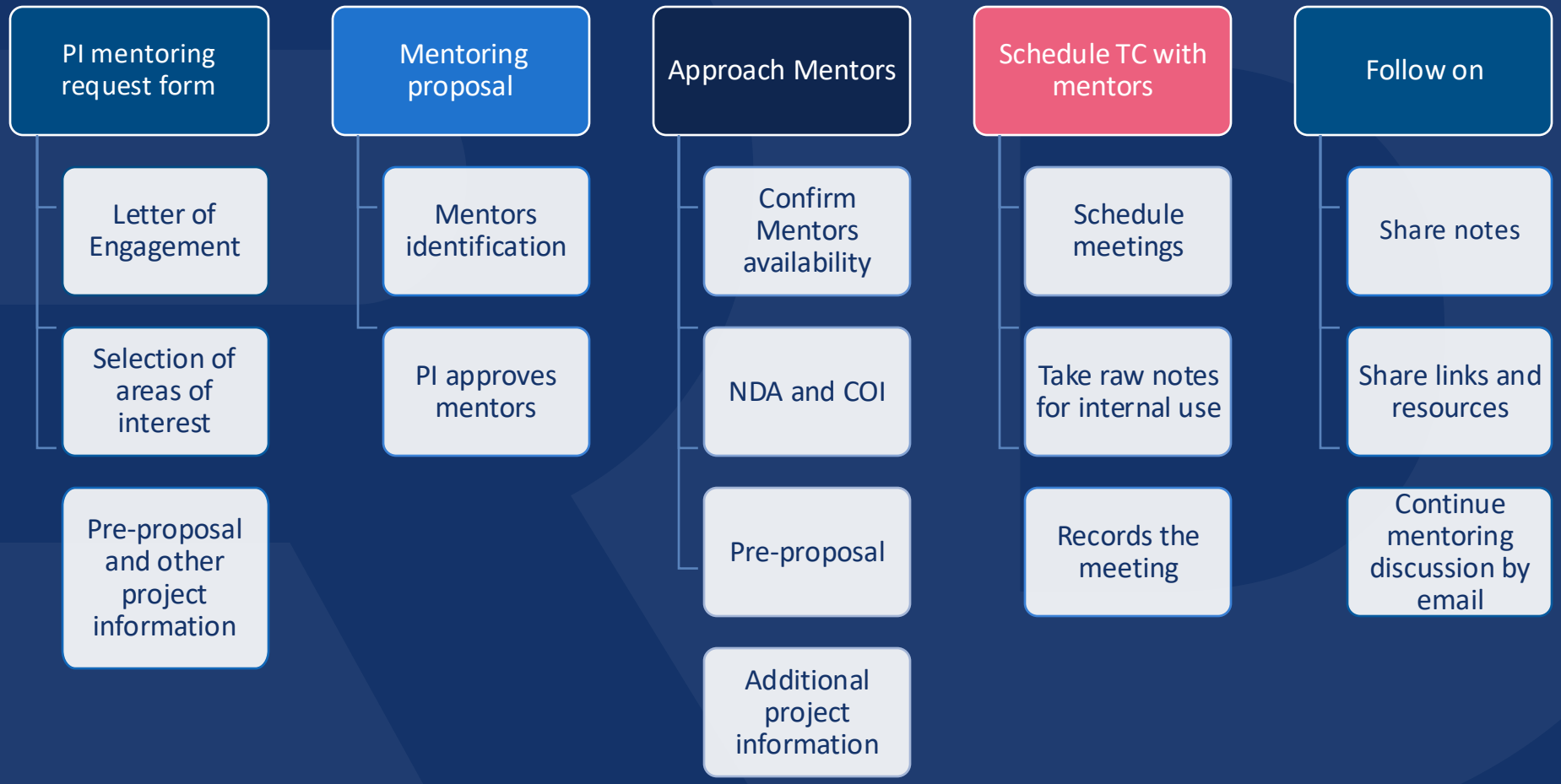
One on one mentoring meetings

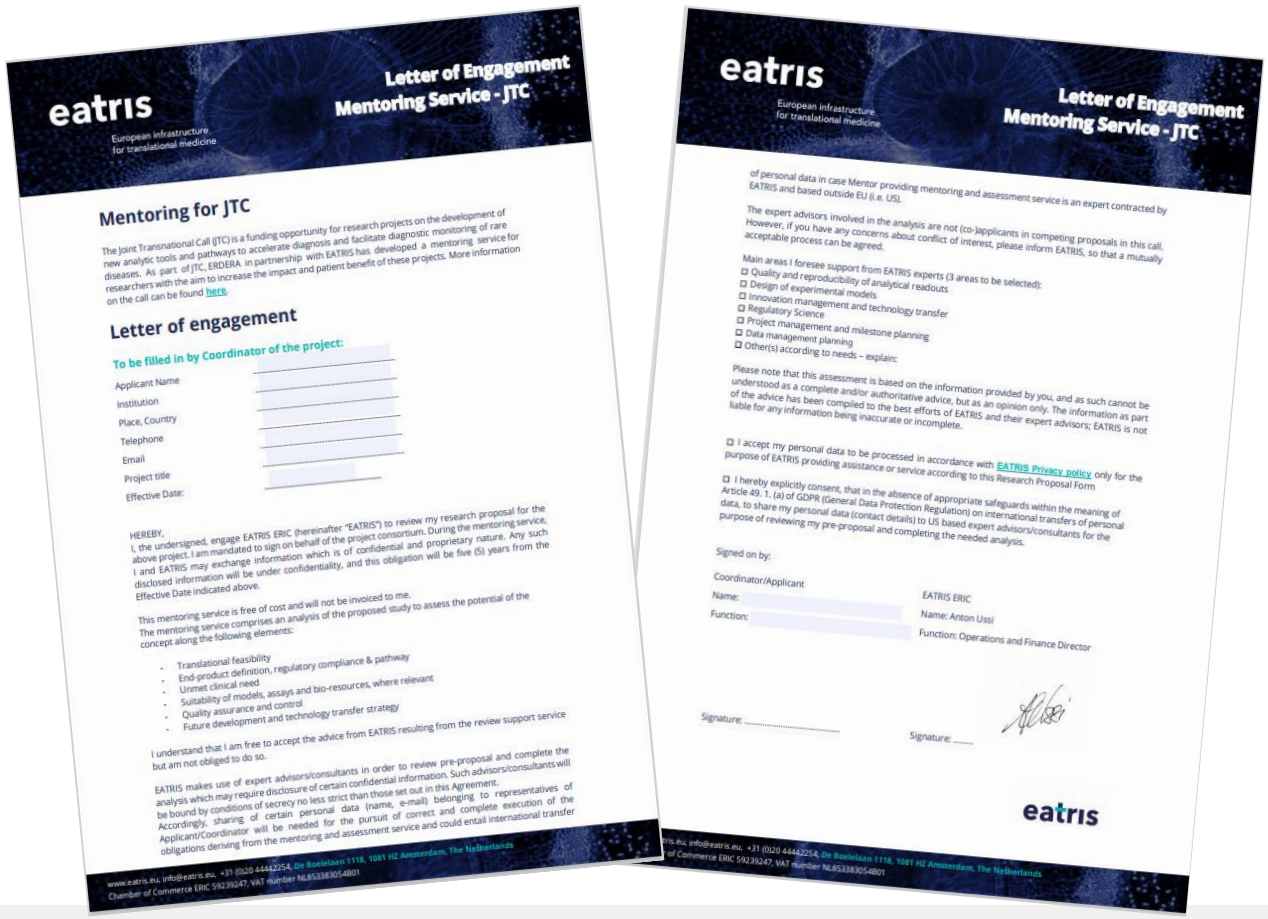


JTC 2026 request form

1. **General Project Information**
2. **Project Thematic Area & modality**
    - Functional validation of variants of uncertain significance
    - Multi-omics and integrative approaches
    - Emerging computational and analytical tools
    - Systems biology and disease-mechanism modelling
    - Integration of clinical, environmental, lifestyle, and sensor data
    - Knowledge graphs, disease maps, and advanced AI models
3. **Current Project Stage (**Research stage)
4. **Specific Mentoring Requests**
5. **Attachment** (collect signed LoE)
6. PI's availability

https://wkf.ms/4i6JclQ

Letter of Engagement



eatris
European infrastructure for translational medicine

Letter of Engagement
Mentoring Service - JTC

Mentoring for JTC

The Joint Transnational Call (JTC) is a funding opportunity for research projects on the development of new analytic tools and pathways to accelerate diagnosis and facilitate diagnostic monitoring of rare diseases. As part of JTC, ERDERA in partnership with EATRIS has developed a mentoring service for researchers with the aim to increase the impact and patient benefit of these projects. More information on the call can be found here.

Letter of engagement

To be filled in by Coordinator of the project:

Applicant Name
Institution
Place, Country
Telephone
Email
Project title
Effective Date:

HEREBY,
I, the undersigned, engage EATRIS ERIC (hereinafter "EATRIS") to review my research proposal for the above project. I am mandated to sign on behalf of the project consortium. During the mentoring service, I and EATRIS may exchange information which is of confidential and proprietary nature. Any such disclosed information will be under confidentiality, and this obligation will be five (5) years from the Effective Date indicated above.

This mentoring service is free of cost and will not be invoiced to me.
The mentoring service comprises an analysis of the proposed study to assess the potential of the concept along the following elements:

- Translational feasibility
- End-product definition, regulatory compliance & pathway
- Unmet clinical need
- Suitability of models, assays and bio-resources, where relevant
- Quality assurance and control
- Future development and technology transfer strategy

I understand that I am free to accept the advice from EATRIS resulting from the review support service but am not obliged to do so.

EATRIS makes use of expert advisors/consultants in order to review pre-proposal and complete the analysis which may require disclosure of certain confidential information. Such advisors/consultants will be bound by conditions of secrecy no less strict than those set out in this Agreement.
Accordingly, sharing of certain personal data (name, e-mail) belonging to representatives of Applicant/Coordinator will be needed for the pursuit of correct and complete execution of the obligations deriving from the mentoring and assessment service and could entail international transfer

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Letter of Engagement
Mentoring Service - JTC

of personal data in case Mentor providing mentoring and assessment service is an expert contracted by EATRIS and based outside EU (i.e. US).

The expert advisors involved in the analysis are not (co-)applicants in competing proposals in this call. However, if you have any concerns about conflict of interest, please inform EATRIS, so that a mutually acceptable process can be agreed.

Main areas I foresee support from EATRIS experts (3 areas to be selected):
- ☐ Quality and reproducibility of analytical readouts
- ☐ Design of experimental models
- ☐ Innovation management and technology transfer
- ☐ Regulatory Science
- ☐ Project management and milestone planning
- ☐ Data management planning
- ☐ Other(s) according to needs – explain:

Please note that this assessment is based on the information provided by you, and as such cannot be understood as a complete and/or authoritative advice, but as an opinion only. The information as part of the advice has been compiled to the best efforts of EATRIS and their expert advisors; EATRIS is not liable for any information being inaccurate or incomplete.

☐ I accept my personal data to be processed in accordance with EATRIS Privacy policy only for the purpose of EATRIS providing assistance or service according to this Research Proposal Form

☐ I hereby explicitly consent, that in the absence of appropriate safeguards within the meaning of Article 49. 1. (a) of GDPR (General Data Protection Regulation) on international transfers of personal data, to share my personal data (contact details) to US based expert advisors/consultants for the purpose of reviewing my pre-proposal and completing the needed analysis.

Signed on by:

Coordinator/Applicant
Name:
Function:

EATRIS ERIC
Name: Anton Ussi
Function: Operations and Finance Director

Signature: .....

Signature: ____

eatris

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Chamber of Commerce ERIC 59239247, VAT number NL853383054B01

https://wkf.ms/4i6JclQ

It must be signed and uploaded before submitting the request form

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GA n°825575

Innovation Management Toolbox

ERDERA European Rare Diseases Research Alliance

Innovation Management Toolbox

Covering key areas essential to translational research success

IMT features

Advanced browser
Filter the search by categories, tags, and geographical scope

Q&A List
Relevant questions on drug development steps

Use Cases
Short videos created by experts on different drug development topics

ERICA Catalogue
Catalogue of services for the Rare disease research community

Collections
Integration of other toolkits, courses and libraries.

Use Cases

- Introduction to Project Management
- Introduction to Innovation Management
- How to meaningfully engage with patients in your research
- Introduction to Data Management

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ERDERA European Rare Diseases Research Alliance

Q&A section

How do I develop my biomarker strategy?

Biomarkers should be introduced at the start of the drug discovery pipeline at target selection stage. It is important to understand the mechanism of action with the target and all the markers involved. This can be established using different multianalyte assays and techniques to identify some key markers that can be monitored once the compound reaches the clinic, using a robust assay that has been developed during the biomarker identification. Source: Gayle Marshall, Lead Scientist for Biomarkers at MDC.

←How do I choose the right disease model?

Why is the Natural History so important in rare diseases? →

Related Resources

BEST (Biomarkers, Endpoints, and other Tools) Resource

Published by FDA-NIH Biomarker Working Group

Discovery and validation of biomarkers to aid the development of safe and effective pain therapeutics: challenges and opportunities

Published by Nature Reviews Neurology

Biomarker Commercialization Review Tool

Published by Biomarker Commercialization (BIC) consortium

Research and Drug Development | Biomarker | Guideline | Biomarker Commercialisation (BIC) Consortium

The BIC Review Tool is designed to improve communication, it allows an "inventors interview"-type of approach, in addition to being a checklist for Technology Transfer Offices and Researchers. It is designed to improve communication between the stakeholders involved in the commercialization process and to facilitate collaboration. The tool is therefore in a completely different format than the Biomarker Commercialization (BIC) Guide. All introductions and explanations are missing by intention since they can already be found in the BIC Guide, Best & Pitfall Practices handbook or the Regulatory Guide.

View this resource | Bookmark this resource

Collections

Orphan drug Guideline ODDG (IRDiRC): Is an interactive tool to guide researchers through the whole process of the rare disease therapies development

Rare Disease Clinical trial Toolbox (ECRIN): Resources organized in a way to guide and help clinical trialists and R&D managers understand the regulations and requirements for conducting trials

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Feedback ERDERA JTC 2025 mentored projects

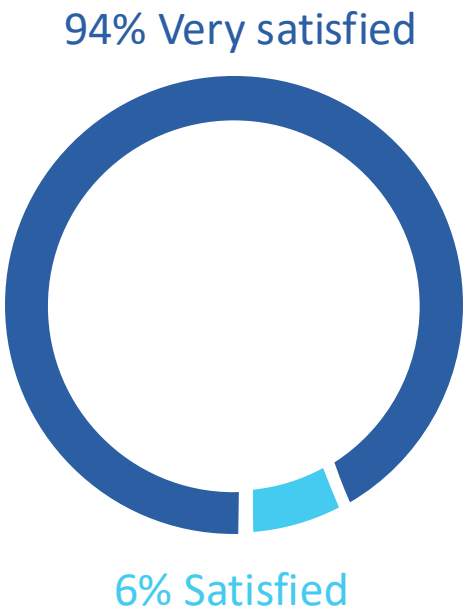
> **Transformative discussions** that identified weaknesses and offered solutions.

> **Clear, specific, and actionable advice** that helped refine proposals.

> **Increased knowledge** on translation strategies, regulatory aspects, and project management.

> **Immediate impact** on proposal quality (scientific rigor, regulatory compliance, structure)

> **Professional, well-prepared mentors** with deep expertise in project details



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GA n°825575

Exploring RD Researchers' Needs for Consultancy Services survey

ERDERA has launched this survey to map in which areas Rare Disease (RD) research community across Europe and beyond need external consultancy support most.

Its findings could help shape a new dedicated ERDERA consultancy service offering specialised advice to projects funded through the partnership and the broader RD research community.

Scan me!

ERDERA European Rare Diseases Research Alliance

European **Rare Diseases**
Research Alliance

Thank you!

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