ERDERA

European Rare Diseases
Research Alliance

ERDERA Research Initiatives for Therapy Development in Rare Diseases

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INSERM, France

Advancement of Treatment for Rare Diseases
16 – 17 of June 2026
The Cyprus Institute of Neurology and Genetics
Nicosia, Cyprus

Co-funded by the European Union

ERDERA has received funding from the European Union's Horizon Europe research and innovation programme under grant agreement N°101156595.
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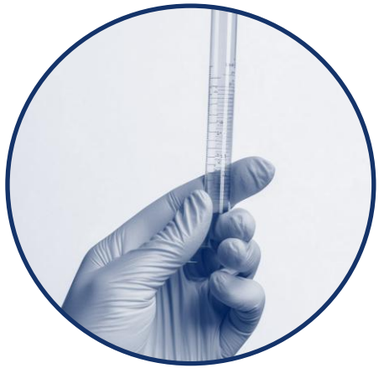
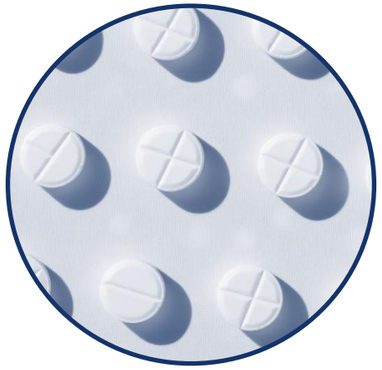
Improving the health and well-being of 30 million people living with a rare disease by making Europe a world-leader in RD research and innovation.



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: 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

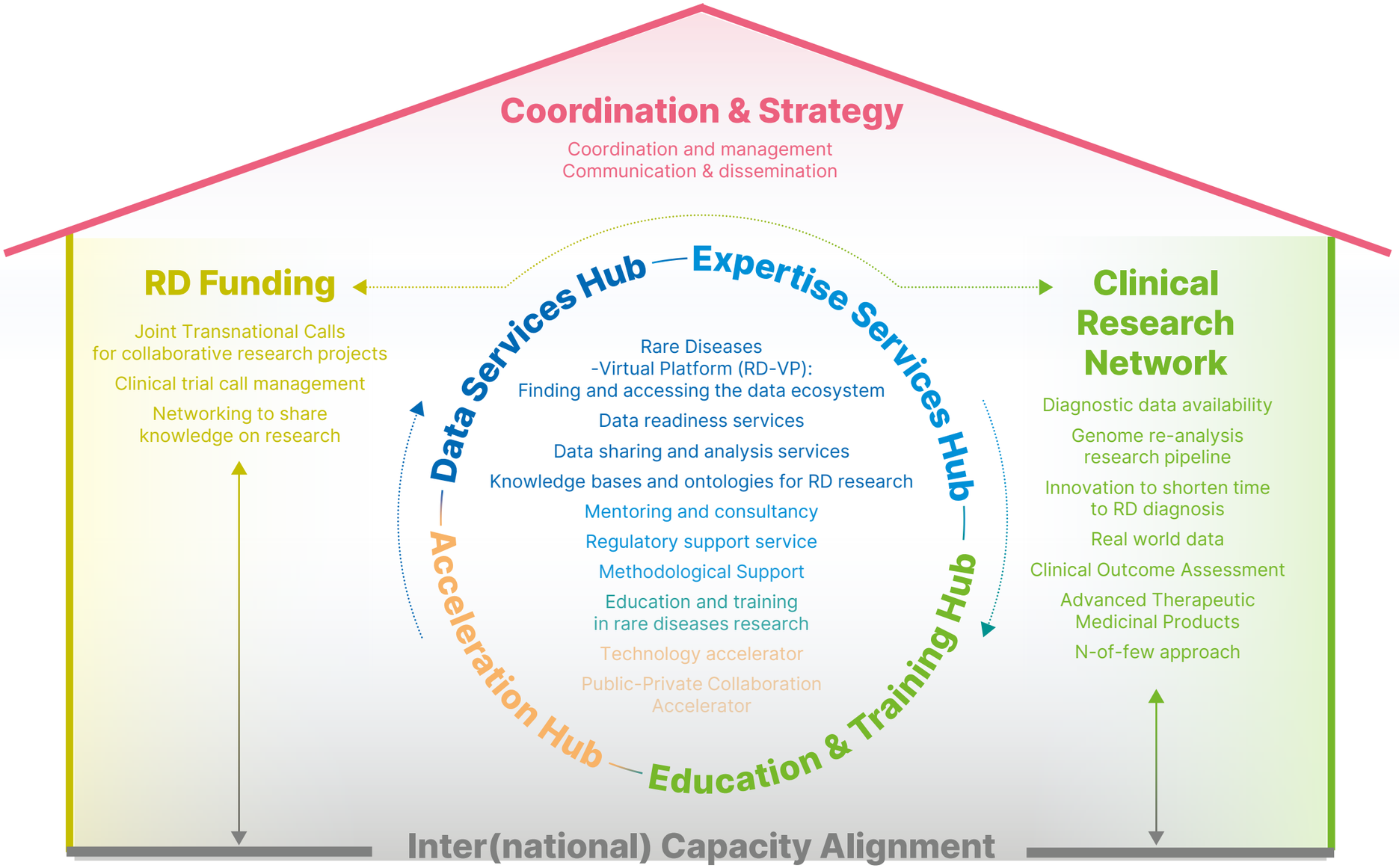
ERDERA spans **25 European Union countries** (except Croatia and Malta) and **numerous associated and non-EU countries**, fostering international cooperation (Australia, Canada, Iceland, Israel, Georgia, Morocco, New Zealand, Norway, Serbia, Switzerland, Türkiye, United Kingdom)

Substantial Financial Commitment

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

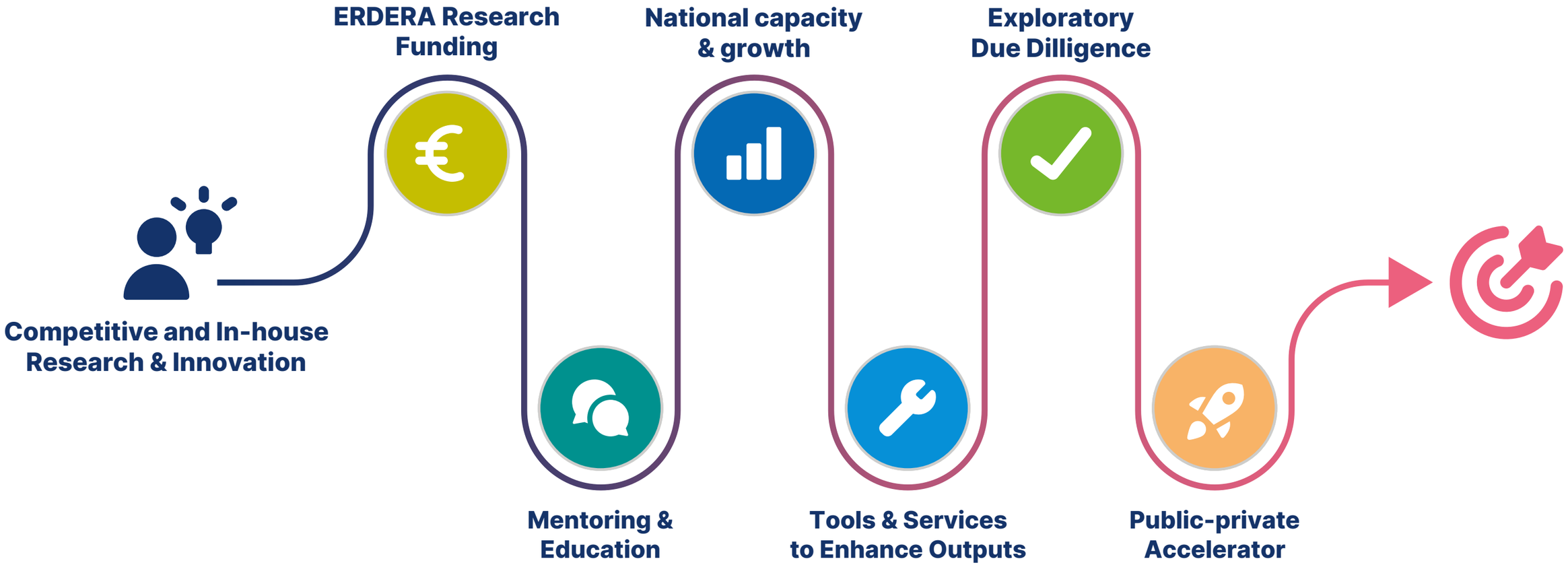
- **€150 million** investment from the European Commission
- Over €230 million contributed by participating countries and institutions
- **Every partner contributes** to our collective actions

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

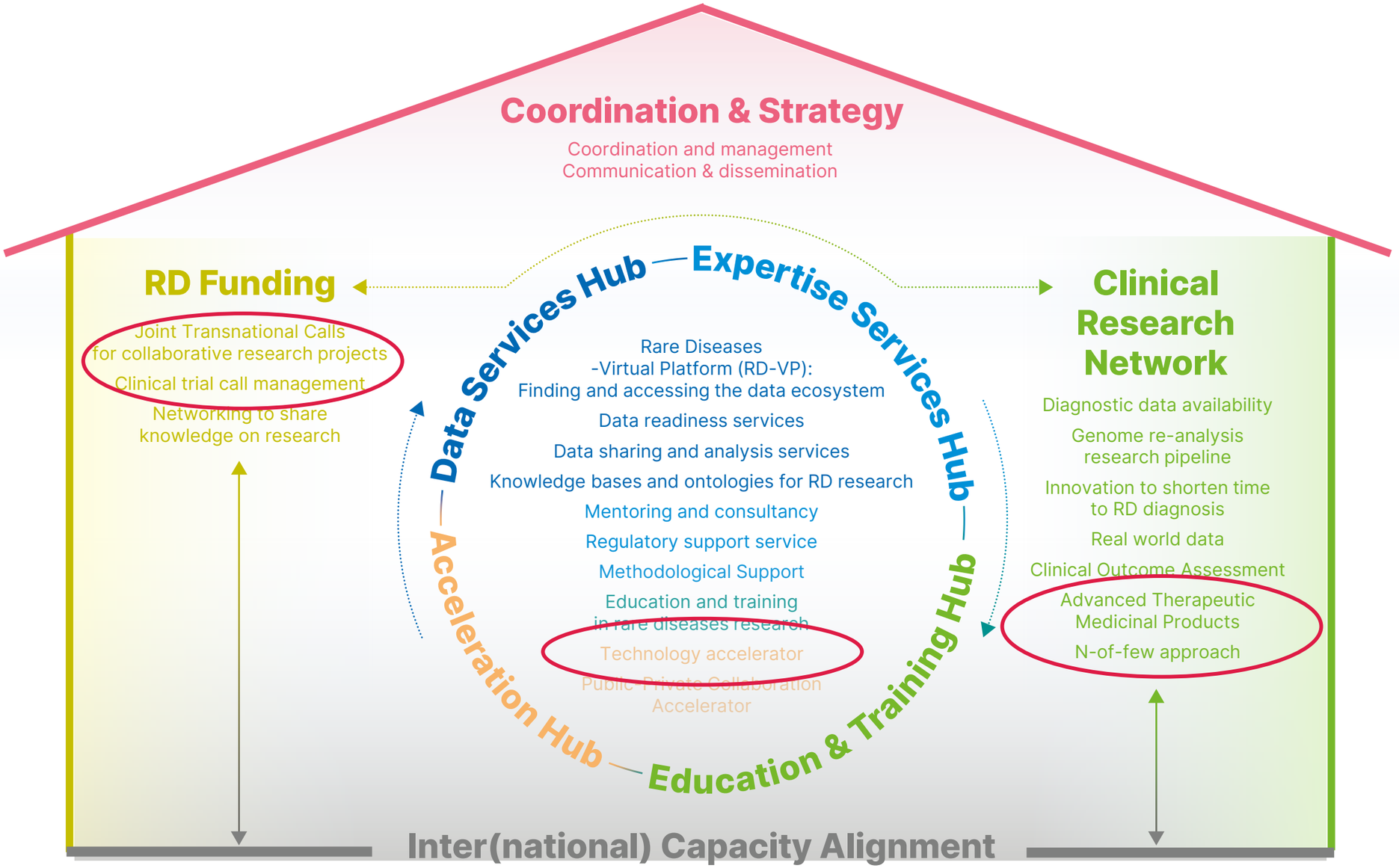


ERDERA European Rare Diseases Research Alliance

ERDERA’s research & innovation support cycle



ERDERA’s approach to Therapies


Coordination & Strategy
Coordination and management
Communication & dissemination
RD Funding
Joint Transnational Calls for collaborative research projects
Clinical trial call management
Networking to share knowledge on research
Data Services Hub
Expertise Services Hub
Acceleration Hub
Education & Training Hub
Rare Diseases -Virtual Platform (RD-VP): Finding and accessing the data ecosystem
Data readiness services
Data sharing and analysis services
Knowledge bases and ontologies for RD research
Mentoring and consultancy
Regulatory support service
Methodological Support
Education and training in rare diseases research
Technology accelerator
Public-Private Collaboration Accelerator
Clinical Research Network
Diagnostic data availability
Genome re-analysis research pipeline
Innovation to shorten time to RD diagnosis
Real world data
Clinical Outcome Assessment
Advanced Therapeutic Medicinal Products
N-of-few approach
Inter(national) Capacity Alignment
NMGs promotion and national alignment
Fostering engagement of underrepresented countries in ERDERA
ERDERA Global Collaboration

Competitive funding

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

Typical success rate: 1st stage vs final funding = 10-12%; 2nd stage vs final funding 35 -50%. Typical overall project budget 1.0 – 1.5 Million €

https://erdera.org/funding/#joint-transnational-call

Launched every year in December with pre-announcement the latest in November

2-stage evaluation process. 3-years projects

A minimum of 3/4 eligible partners and a max. of 6 per project (can be extended to 8 according to specific conditions)
PAOs participation is financed

Call for Proposals 2026

Resolving unsolved cases in rare genetic and non-genetic diseases through variant validation and new technological approaches

Launch 10 December

Pre-announcement now available

Webinar:
16 December,
15:00–17:00 CET

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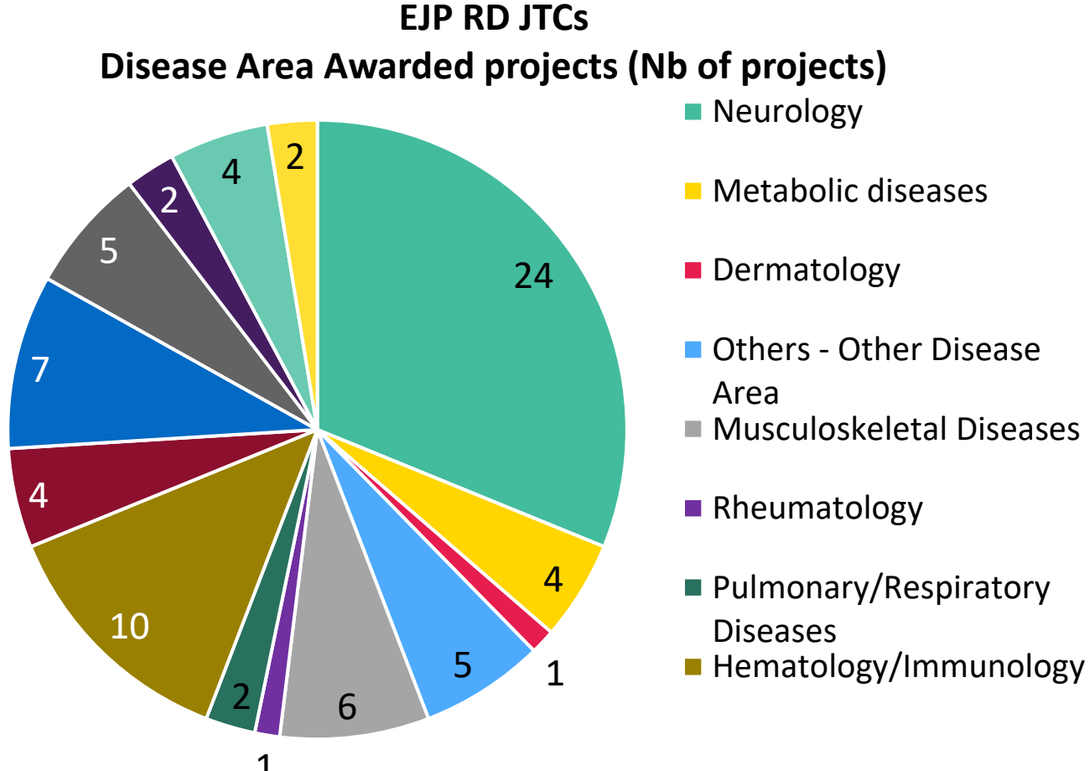
Joint Transnational Calls in EJP RD

- **5 calls launched:**
  - **JTC 2019 –** Research projects to accelerate diagnosis and/or explore disease progression and mechanisms of rare diseases
  - **JTC2020 –** Pre-clinical research to develop effective **therapies for rare diseases**
  - **JTC2021 –** Social Sciences and Humanities Research to Improve Health Care Implementation and Everyday Life of People Living with a Rare Disease
  - **JTC2022 –** Development of new analytic tools and pathways to accelerate diagnosis and facilitate diagnostic monitoring of rare diseases
  - **JTC2023 –** Natural History Studies addressing unmet needs in Rare Diseases

77 funded projects

More than **104million euros**

➔For more information on past calls (call documents, etc.):
https://www.ejprarediseases.org/past-funding-opportunities/



Co-funded by the European Union

Joint Transnational Call 2025

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

27 Countries

35 Funders

Secretariat DLR

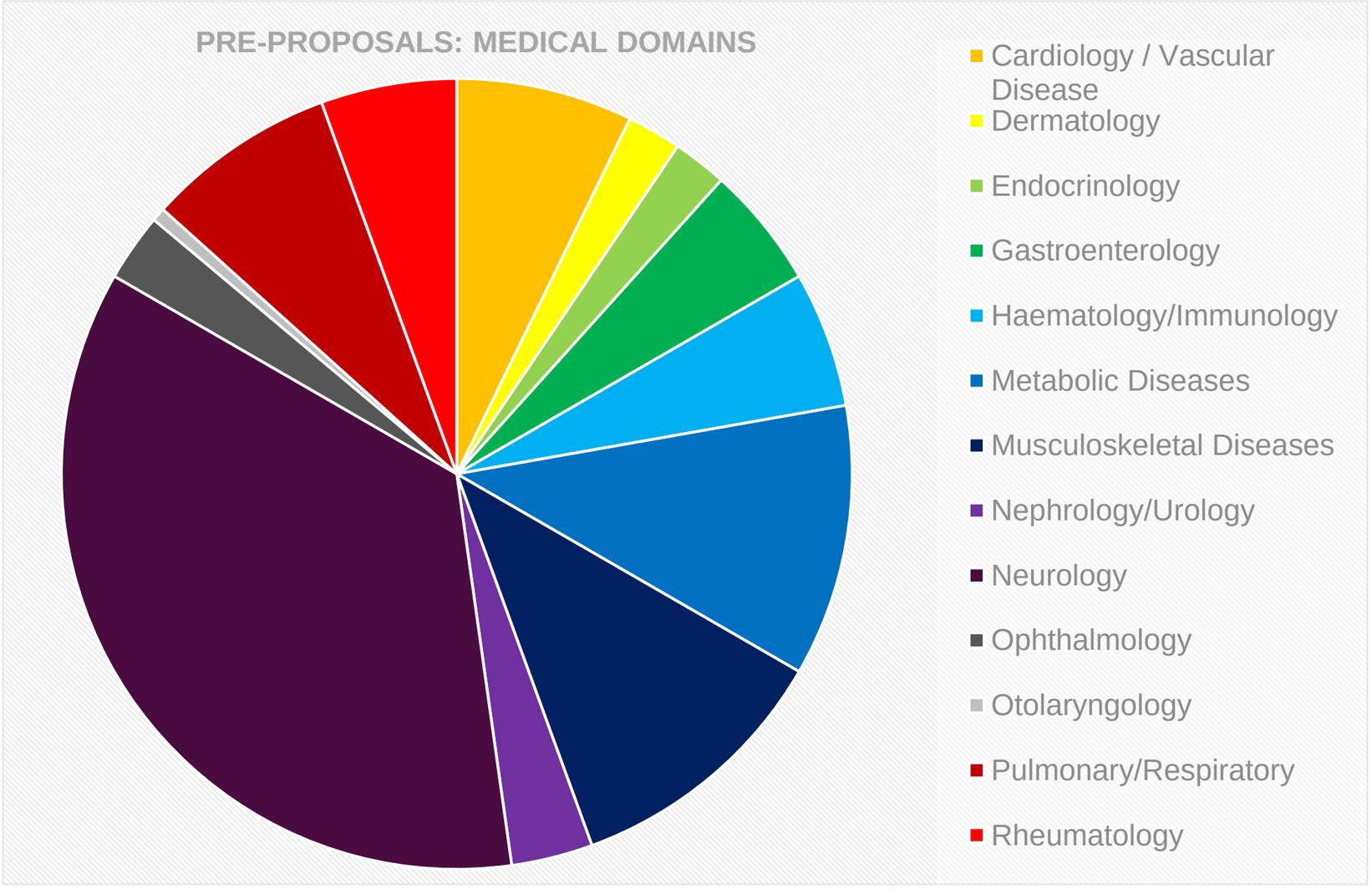
33 M€ Committed

Pre-proposals
183
260 M€ Requested

Eligible
164
238 M€ Requested

Full proposals
48
72 M€ Requested

Funded Consortia
18
28.9 M€ funded*



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*** Including 3.24 M€ contribution from the European Commission**

Clinical Trials Call

Clinical Trials Call funds controlled clinical research studies undertaken in humans to establish or confirm the safety and effectiveness of therapeutic interventions. These will benefit from ERDERA support for regulatory and methodological aspects.

Clinical Trials Call

30 million € budget from the European Commission + possible additional funds from national funding bodies

Definition of call topic and rules in 2025. Opening of the call in 2026. Final decision by end of 2027 and funding of clinical trials between 2028-2031 with possible extension to 2034.

Driven by patients' needs from inception

Optimised support: How do we get there?

- 10 months of intense preparations:
  - Expert groups composed of multiple stakeholders representing all interests & views (patients, researchers, industry, regulators, clinicians, infrastructures, funders, etc.)
  - Interviews with specialist/expert: patients, industry, clinical trial infrastructures, regulators, etc.
  - Direct discussion & inclusion of the European Medicines Agency and national regulators in the scientific advice process during call
  - Inclusion of expert with over 30 years of experience in clinical trials & CRO in the coordination team

There is a solution for every known problem

Bottleneck identified	Proposed solution
Budget fragmentation & public funding mechanism	Central budget, operated by single funder experienced in the implementation of “industry-like” clinical trials. Project time of min. 5 years. Payment based on delivery of milestones.
The burden of investigator-sponsor role	Professional support/infrastructure with in-depth knowledge of multi-national CT management, regulatory, legal & ethics support provided for free to applicants
Logistics of drug procurement & drug supply	Evaluation criteria & supporting services ensuring that this aspect is acknowledged & covered from the proposal stage
Clinical trial design not adapted to patient needs	PPIE support from the start/during the proposal preparation stage. Possibility to revise/adapt the design.
Clinical trial design not adapted/acceptable by regulator	Dedicated (free of charge) service to accompany applicants + direct involvement of EMA and national scientific advice
Administrative heterogeneity	Professional support/infrastructure with in-depth knowledge of multi-national CT management/homogenous templates/centralisation of parts of the trial (e.g. use of common data collection tools & provider)

ERDERA Technology Accelerator
&
Clinical Research Network Therapy Pole

ERDERA European Rare Diseases Research Alliance

Derisking and scaling in vivo ATMP therapies through an open-access European expertise network.

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The WP21 Value Chain: An integrated assembly line for Advanced Therapy Medicinal Products.

T21.1

Harmonise Assays

Harmonised rAAV manufacturing and analytical assays.

T21.2

Optimise Synthesis

RNAs for therapeutics: optimised design, synthesis, and scalable production.

T21.3

Benchmark Systems

mRNA delivery: benchmark LNPs, EVs, biohybrids; standardise evaluation protocols.

T21.4

Comparative Evaluation

Gene editing: comparative evaluation of CRISPR, base & prime editing; focus on RDs in blood, blood, CNS, retina, liver, and muscle.

T21.5

Immune De-risking

Therapy response: advanced immune monitoring and de-risking strategies.

Delivering translation-ready pipelines to the clinical frontier.

T21.4 (Gene Editing)

Disease-Specific Strategies

Developing targeted interventions for SCD, FA, ALS, RP4, DMD, etc.

Precision editors identified as the preferred approach for sensitive tissues.

T21.5 (Therapy Response)

High-Resolution Immune Profiling

Establishing spectral flow and multi-omics profiling.

Proof-of-concept achieved for immune mitigation (construct redesign, Treg approaches).

Creation of standardised, translatable technology pipelines.

Ready for WP11 Selection & Clinical Advancement.

Building the Consensus Engine for ATMP Prioritisation

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Task 11.1 (Lead: UKHD) • Refining the framework for rare diseases suitable for Advanced Therapy Medicinal Products.

Driven by the landmark 'Prioritisation of Rare Diseases for ATMP Development' workshop, bridging clinical reality, scientific viability, patient needs, and industry mechanics into a unified framework.

The Two-Step Prioritisation System

Processing a broad landscape into highly specific, actionable development targets.

Systematic Routing to ATMP Technical Platforms

Task 11.2 (Lead: GNT) • Adapting and selecting optimal technological strategies.

The decision-tree approach ensures that every selected disease category is systematically and rapidly linked to the most viable ATMP strategy and technical platform, **eliminating ad-hoc R&D bottlenecks.**

WP12: Academic ASO Platform – Objectives & Framework

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Establishing the infrastructure to design, develop, and validate personalised **antisense oligonucleotide** (ASO) therapies for ultra-rare diseases.

Decision Frameworks (T12.2)

Benefit-Risk Assessment

Refined criteria for individualised therapies, establishing clear rules for treatment continuation and discontinuation.

Ethical Consent

Coordinated consensus outputs for unified patient communication and informed consent, particularly tailored for neurodevelopmental n-of-1 trials.

Outcome Toolbox

Domain Prioritisation

International consensus framework establishing prioritised outcome domains specifically tailored for n-of-1 and n-of-few trials.

Natural History 'Run-In'

Specialised frameworks to establish individual patient baselines, ensuring statistically sound efficacy decisions for single-patient scenarios.

Operational Readiness (T12.3 & T12.4)

Master Protocols

Harmonised, adaptable study designs for aggregated n-of-1 treatments to guarantee consistent cross-site data analysis.

Institutional Alignment

'Operational readiness' criteria ensuring clinical sites meet all legal and technical demands for national named-patient administration frameworks.

WP12 Progress: Accelerating Case Studies & Regulatory Alignment

Advancing lead programmes toward first-in-human applications while shaping European regulatory pathways.

In Vitro Screening & Assays

Ataxia Telangiectasia (A-T) [Success]

Validated efficacy and toxicity screening for ASOs targeting deep-intronic mutations.

POLR3A Leukodystrophy [Success]

Evaluated 9 ASOs via mini-gene assays; 2 lead candidates successfully restored wildtype splicing.

In Vivo Safety (Current Phase)

Advancing 3 lead ASO programmes into comprehensive safety testing using rat in vivo models.

First-in-Human Application

Preparing for clinical administration of ERDERA treatments via established master protocols.

The "Platform Approach"

High-level dialogue with the EMA and Dutch MEB validating the overarching platform strategy for ASOs.

International Consensus

Shaping global regulatory pathways for ASO and base-editing technologies through major EU-coordinated workshops (1,000+ participants).

Clinical Translation

Creating the operational blueprint to translate complex regulatory concepts into practical, centre-level operations for project Years 4–7.

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Support services, tools & training

Expertise Service Hub

MENTORING & CONSULTANCY

Mentoring Programme:

32 projects accompanied with over 30 mentors, handbook & tracking platform

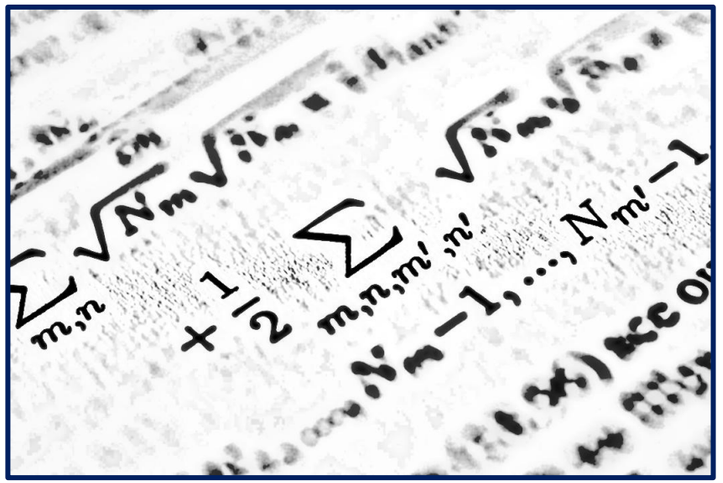
Consultancy service: encompassing overall expertise available in ERDERA tailored to users needs

REGULATORY

Regulatory Support Group:

20 members, representing regulators, academia, industry, patients, non-for profit

Guidance on: regulatory requirement for pre-clinical & clinical research, data use, privacy, samples, etc.



METHODOLOGY

Clinical studies support:

Methodologies for multivariate, hierarchical, incomplete data and federated data analysis

RD trial methodology with initiation of surrogate endpoints evaluation

Handling missing data

Expert Mentoring & Innovation Management Toolbox for RD research projects

- In collaboration with eatris
  European infrastructure for translational medicine
- **What is this?** → Service for researchers planning a project that has **translational potential** for RDs
- **Why?** → to increase the impact of projects by providing tailored expertise along each step of the translational pathway
- **When?** → in the pre-proposal stage OR after having received funding
- **How?** → after preliminary discussion and (if needed) confidentiality agreement, EATRIS identifies the most suitable experts that advise and/or accompany the proposal/project
- **Which expertise?** → You can get support on the following topics: Translational feasibility/ Regulatory compliance/Product classification/Quality assurance and control/Intellectual property strategy/Suitability of analytical readouts/Manufacturing/Therapy development
- **How much does it cost?** → service is provided for free
- **Interested? More info** → contact us: coordination@erdera.org & **https://www.ejprarediseases.org/mentoring/**
- **IMT: https://imt.ejprarediseases.org → < 400 resources and specific use cases curated to maintain database updated**

Expert Mentoring & Innovation Management Toolbox for RD research projects

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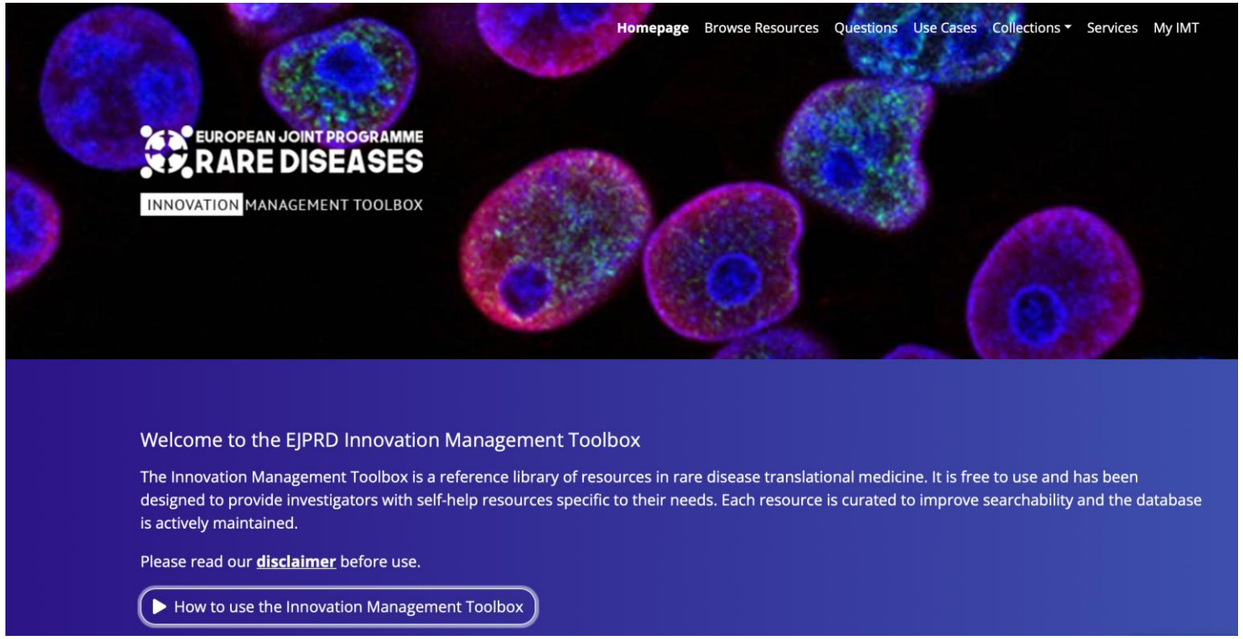
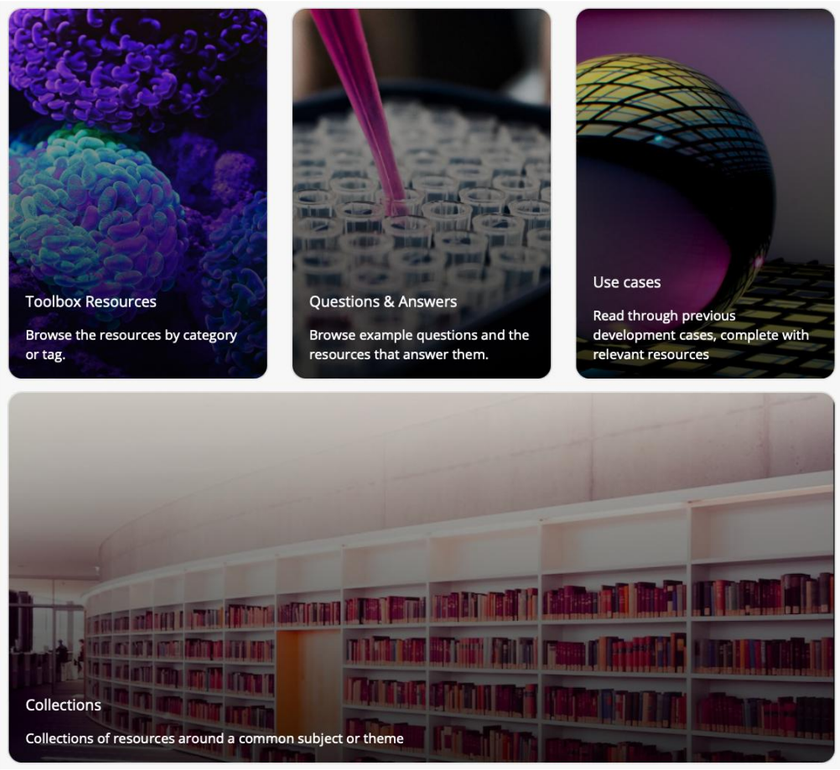
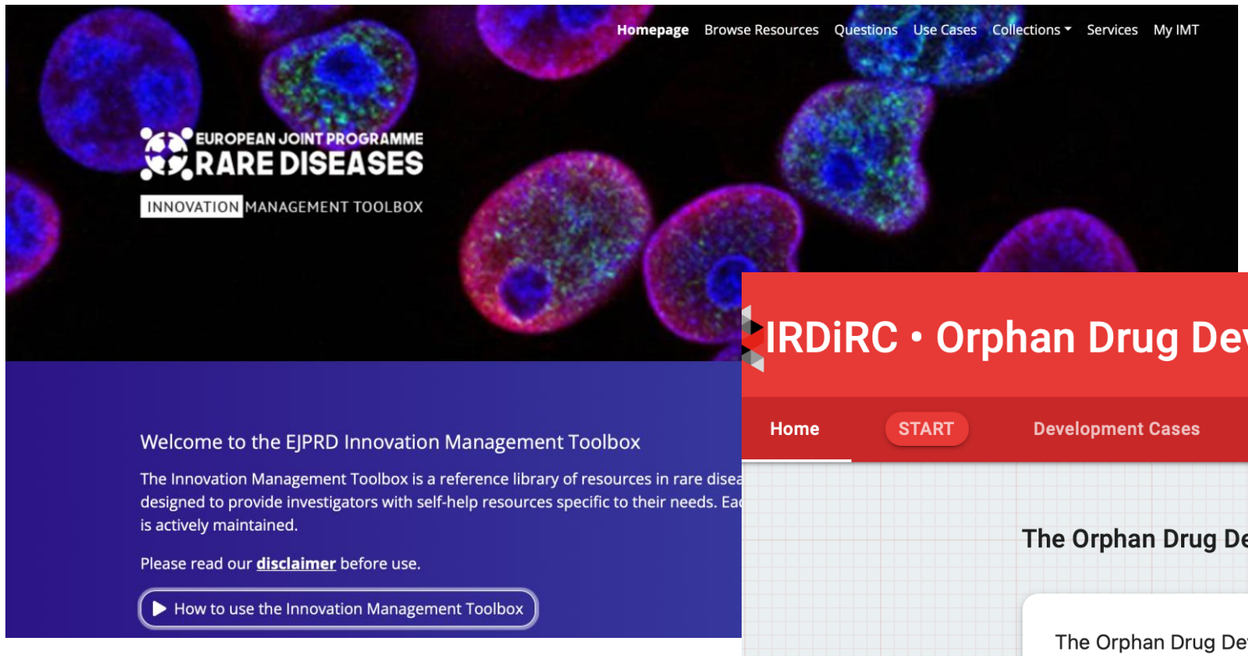




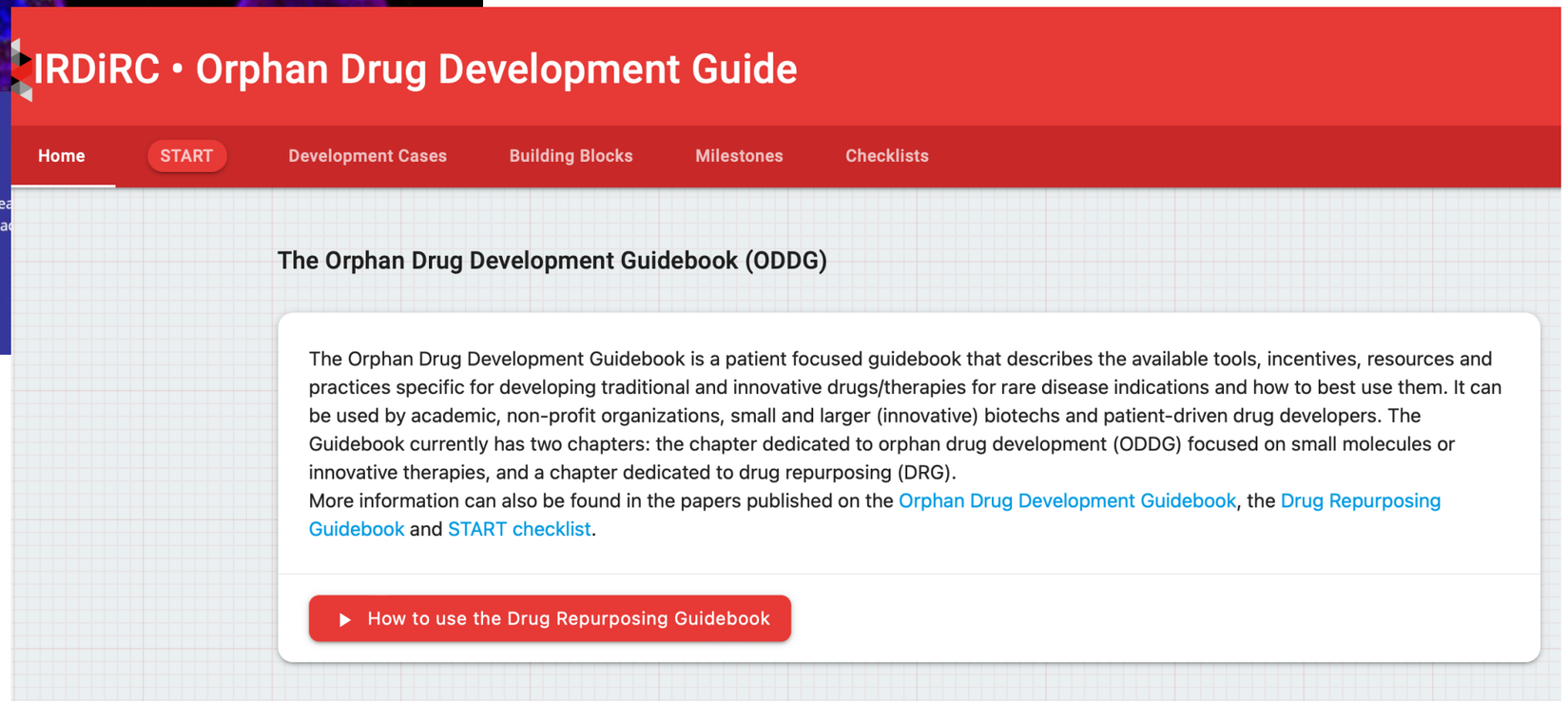
- Library of more than 400 resources
- Free and accessible to any person
- For the ones interested in translational medicine and innovation in RDs
- Resource is curated to improve searchability and the database is actively maintained

Expert Mentoring & Innovation Management Toolbox for RD research projects

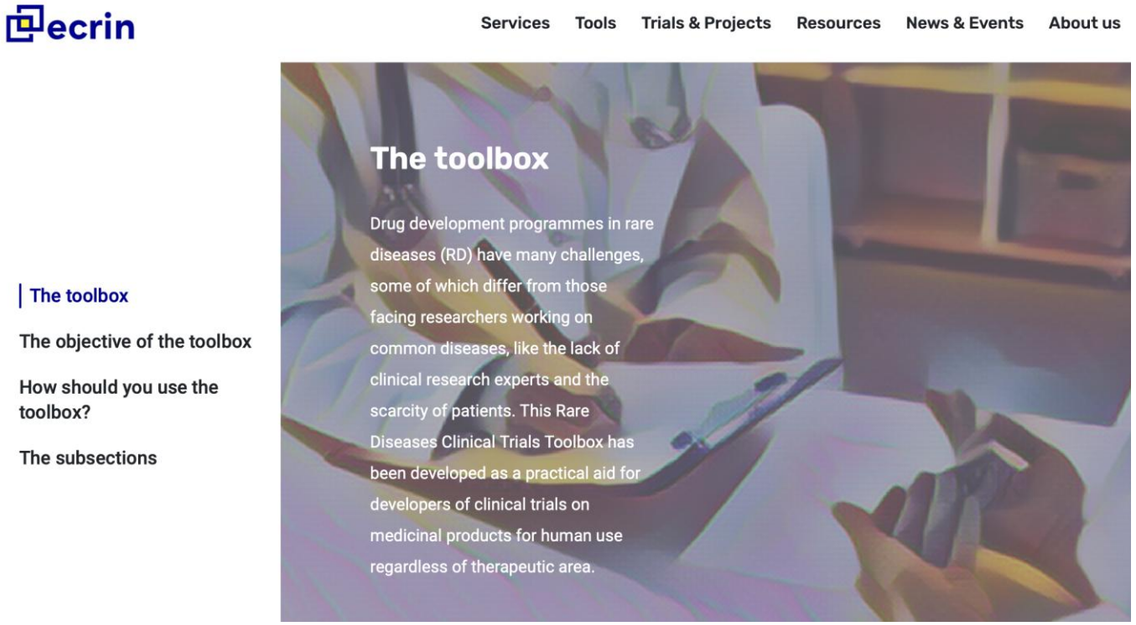


- In collaboration with IRDiRC INTERNATIONAL RARE DISEASES RESEARCH CONSORTIUM

https://orphandrugguide.org



Clinical Trials Toolbox & Methodology Support



https://www.ejprarediseases.org/rare-diseases-clinical-trials-toolbox/

https://www.ejprarediseases.org/clinical-trials-methodology/

Basic Courses | Intermediate Courses | Advanced Webinars | Videos

Cover:

- Recommended **courses for fundamentals in CTs** (not developed by EJP RD)
- **Intermediate courses** aim to provide a specialized statistical training to fill the knowledge gap in clinical trial methodologies
- **Advances webinars** addressed to people willing to conduct clinical research in rare diseases with the objective to train them in terminology, communication and understanding of RD clinical trial methodology.

Some topics covered: randomisation/composite endpoints/statistical evaluation of surrogate endpoints/challenges with master protocols, etc.

Public-Private Collaboration Accelerator (PPCA)

ERDERA European Rare Diseases Research Alliance

ABOUT | LIVING WITH RARE | RESEARCH SERVICES | NEWS & UPDATES | RESOURCES | CONTACT US

HOME > ERDERA ACCELERATOR

The **ERDERA Public-Private Collaboration Accelerator (PPCA)** empowers researchers to turn promising translational ideas into investor-ready projects able to improve the lives of people living with rare diseases. By combining tailored scientific guidance with access to a unique European ecosystem of public and private partners, we help you de-risk and advance your work at the discovery and pre-clinical stages.

Official Opening for submissions on January 2026

TRAINING & EDUCATION

2400 stakeholders trained

Diagnosing & Treating Rare Diseases

Diagnosing Rare Diseases: from the Clinic to Research and back – **1,021 participants**

Innovative Therapies and Personalised Medicine for Rare Diseases – **289 participants**

From Lab to Clinic: Translational Research for Rare Diseases – **190 participants**

Health Data, Ethics & Regulatory Frameworks – **111 participants**

FAIR Data Training

Understanding & value of FAIR

FAIR Guiding principles

Advanced concepts & Tools

300 participants, XX countries

Paediatric Training

27 young participants (ages 12–18)

From **7 Young Persons' Advisory Groups (YPAGs)**: Portugal, UK, Albania, Italy, Greece, Ireland

EURORDIS Open Academy

4-day international training with **two tracks:** *Medicines R&D* *Scientific Innovation & Translational Research*

70 participants (60 patient advocates, 10 researchers)

31 countries, >52 rare diseases

15 expert faculty members delivering interactive sessions

Diagnostic Research

3 focused training sessions – **~400 participants** total

Submitting genomic and clinical data for re-analysis – **133 participants, 26 countries**

Clinical annotation using HPOs – **114 participants**

Advancing diagnostics in underrepresented countries – **150 participants, 30 countries**

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To the Brilliant Minds and Big Hearts Behind ERDERA Coo

Inserm
La science pour la santé
From science to health

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Coordinator

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Assistant Coordinator

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Project Manager RD fundings– Expertise Services Hub

Pauline Adam
Project Manager Global collaborations

Clément Moreau
Project Manager National Alignment

Başak Uysal
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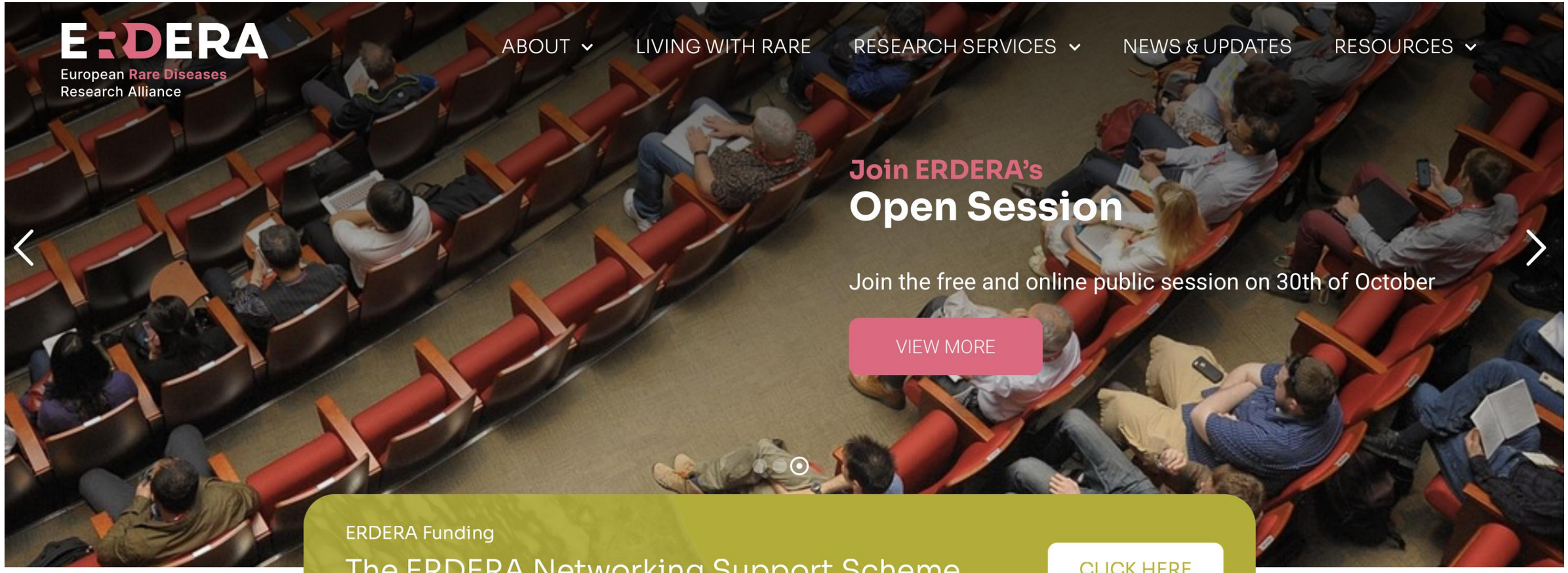

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European **Rare Diseases** Research Alliance

Thank you!

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