



European **Rare Diseases**  
Research Alliance

# Research funding opportunities and ERNs

**Daria JULKOWSKA**  
ERDERA coordinator  
INSERM, France

ERICA ERN Research Conference  
11 – 13 of December 2024  
Udine, Italy



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.  
Views and opinions expressed are those of the author(s) only and do not necessarily reflect those of the European Union or any other granting authority, who cannot be held responsible for them.

# Why and what is ERDERA?

## Why a partnership for rare diseases

- ▶ There are more than **7000** different rare diseases, affecting **30 million** people in Europe.  
Each of us knows someone in their close environment, family or work, living with a rare disease, sometimes we don't even know they have such a disease.
- ▶ For those people, in Europe, on average, **it still takes 4 years to get diagnosed**. During that time, they may be misdiagnosed and shuffled between doctors without clear answers.
- ▶ When the diagnosis finally comes, they find that **no treatment exist for over 90% of rare diseases**—or if they do, they might not be available in their home country.

## ERDERA's Mission

ERDERA aims to change this by **bringing together the strengths of academia, industry, and healthcare to make diagnosis faster, treatments more accessible, and quality of life better for people living with rare diseases**. With ERDERA, we harness the power of research and technology, and we actively engage people living with these diseases, so their needs drive our mission.



**178 Organisations**

**40 funders**

**81 research performing organisations**

**9 patients' organisations**

**3 research infrastructures**

**22 private for-profit partners (industry & SME)**

**23 other (univ, hospital, non-profit, public administration)**

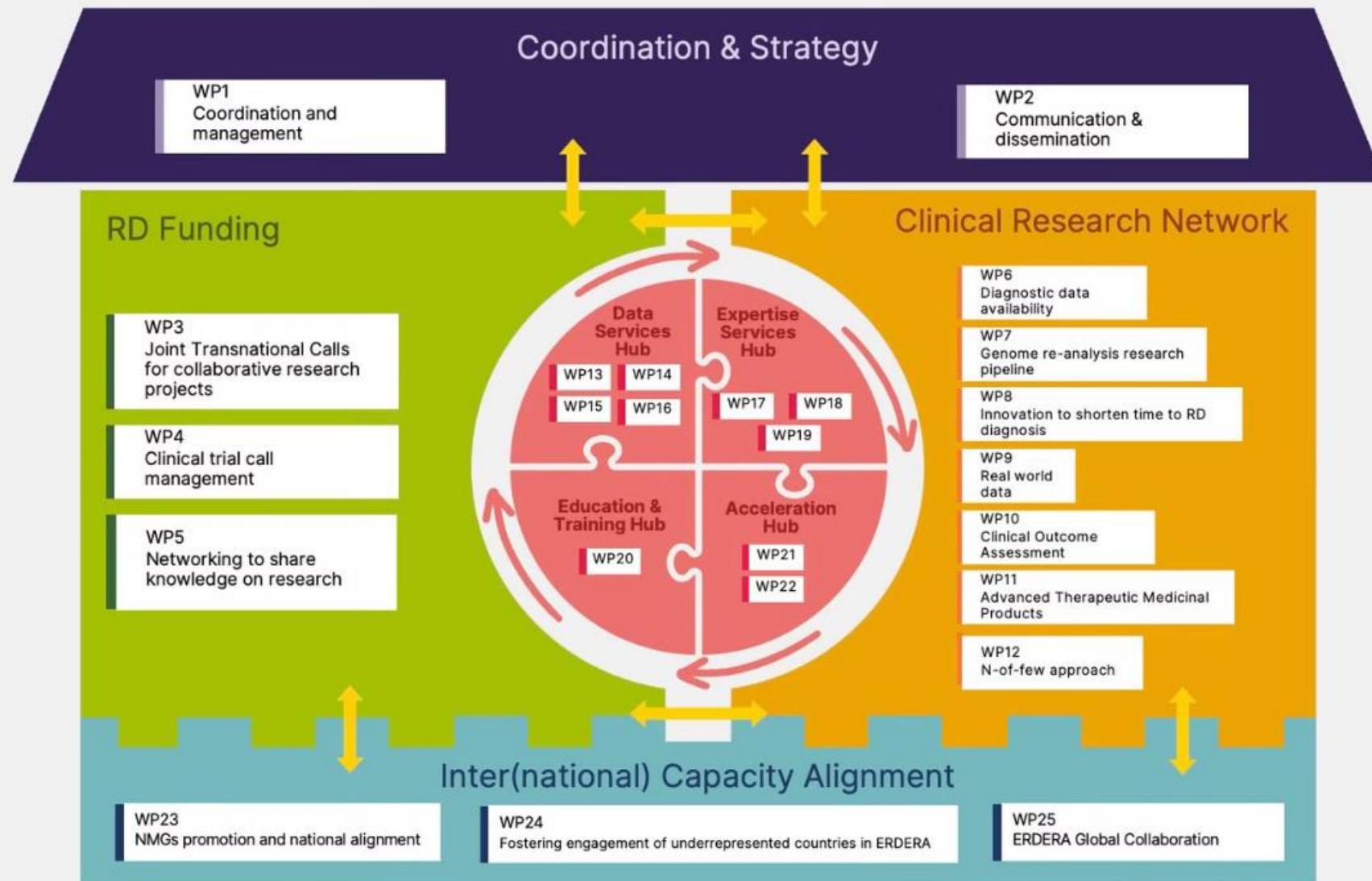
**37 Countries**

**26 EU member states**

**8 associated countries\***

**3 non-EU\***

\*at the time of ERDERA proposal submission



WP13  
Rare Diseases-Virtual Platform (RD-VP): Finding and accessing the data ecosystem

WP14  
Data readiness services

WP15  
Data sharing and analysis services

WP16  
Knowledge bases and ontologies for RD research

WP17  
Mentoring and consultancy

WP18  
Regulatory support service

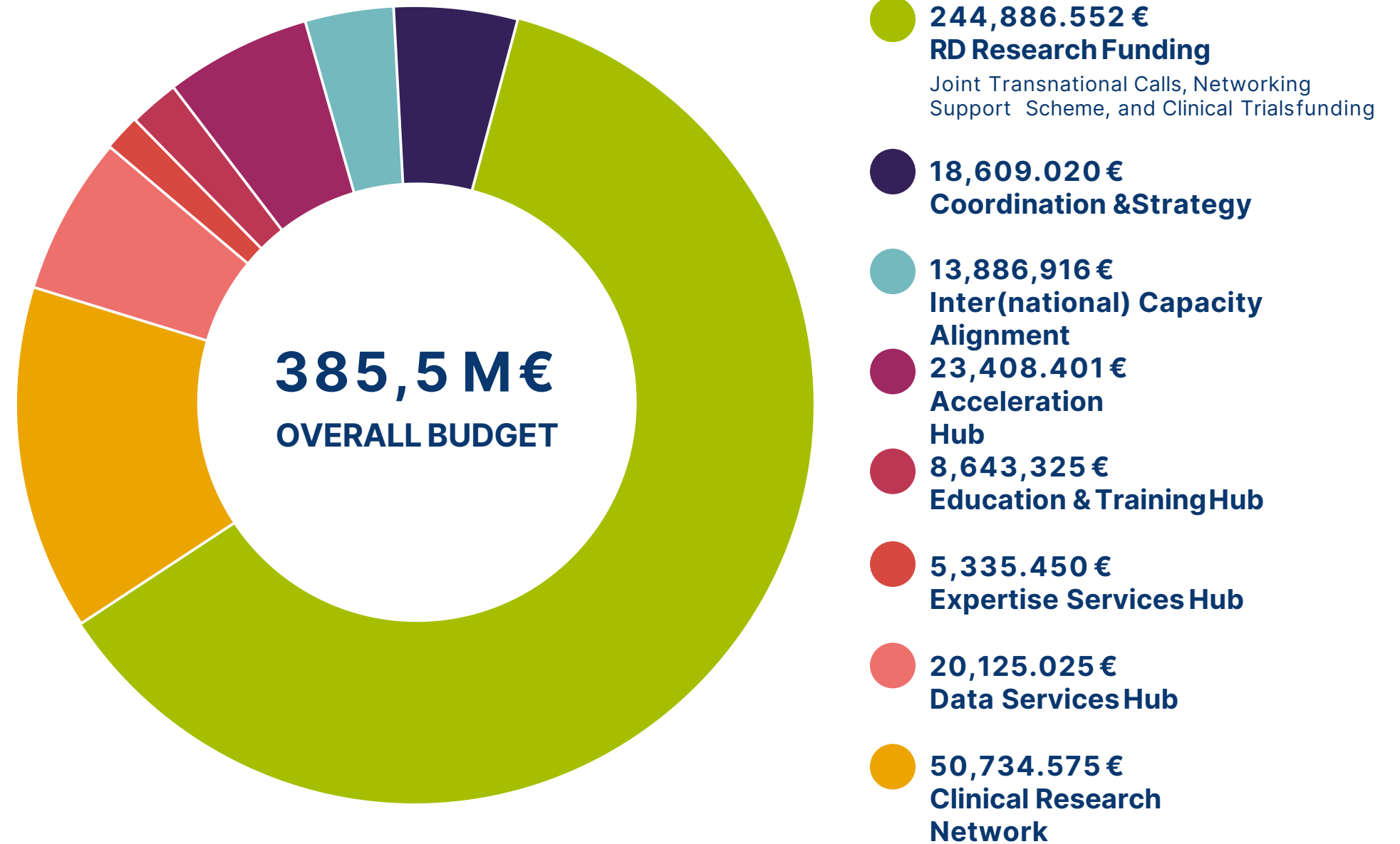
WP19  
Methodological Support

WP20  
Education and training in rare diseases research

WP21  
Technology accelerator

WP22  
Public-Private Collaboration Accelerator

# Total Budget\* per Work Stream



\*estimated for **7 years**, includes:

- (1) associated partners,
- (2) in-kind and in-cash contributions, and
- (3) 150 M€ of European Commission requested contribution

# What are the opportunities of collaboration that ERDERA can offer?



## Yearly Joint Transnational Calls

These calls support **multinational research projects** that are of broad scope. Topics include improving diagnosis, developing new therapies, and understanding the social impact of rare diseases.



## Networking Support Scheme

This scheme offers **small grants** (30 000 €) **to support events or workshops** aimed at fostering knowledge sharing and community building.



## Multinational Clinical Trials Call

A call for **multinational clinical trials** is expected in 2026.



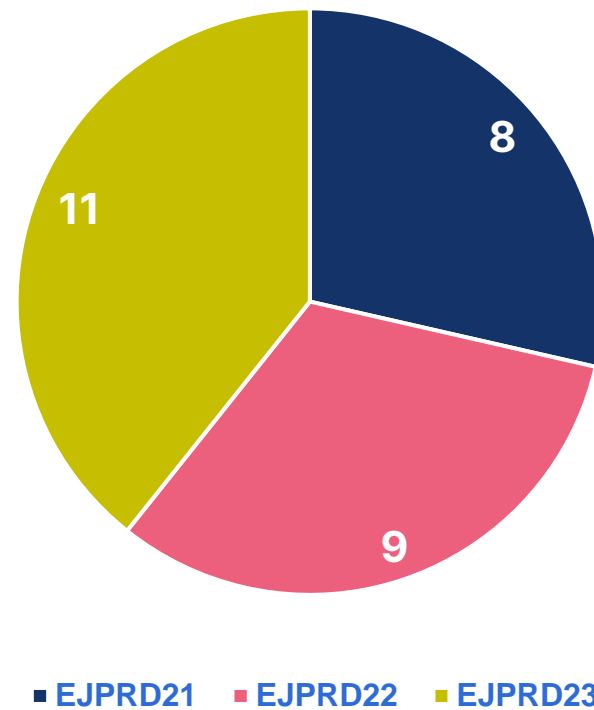
## Direct Collaboration Opportunities

**Collaborate directly with ERDERA's research teams** involved in "in-house" research.

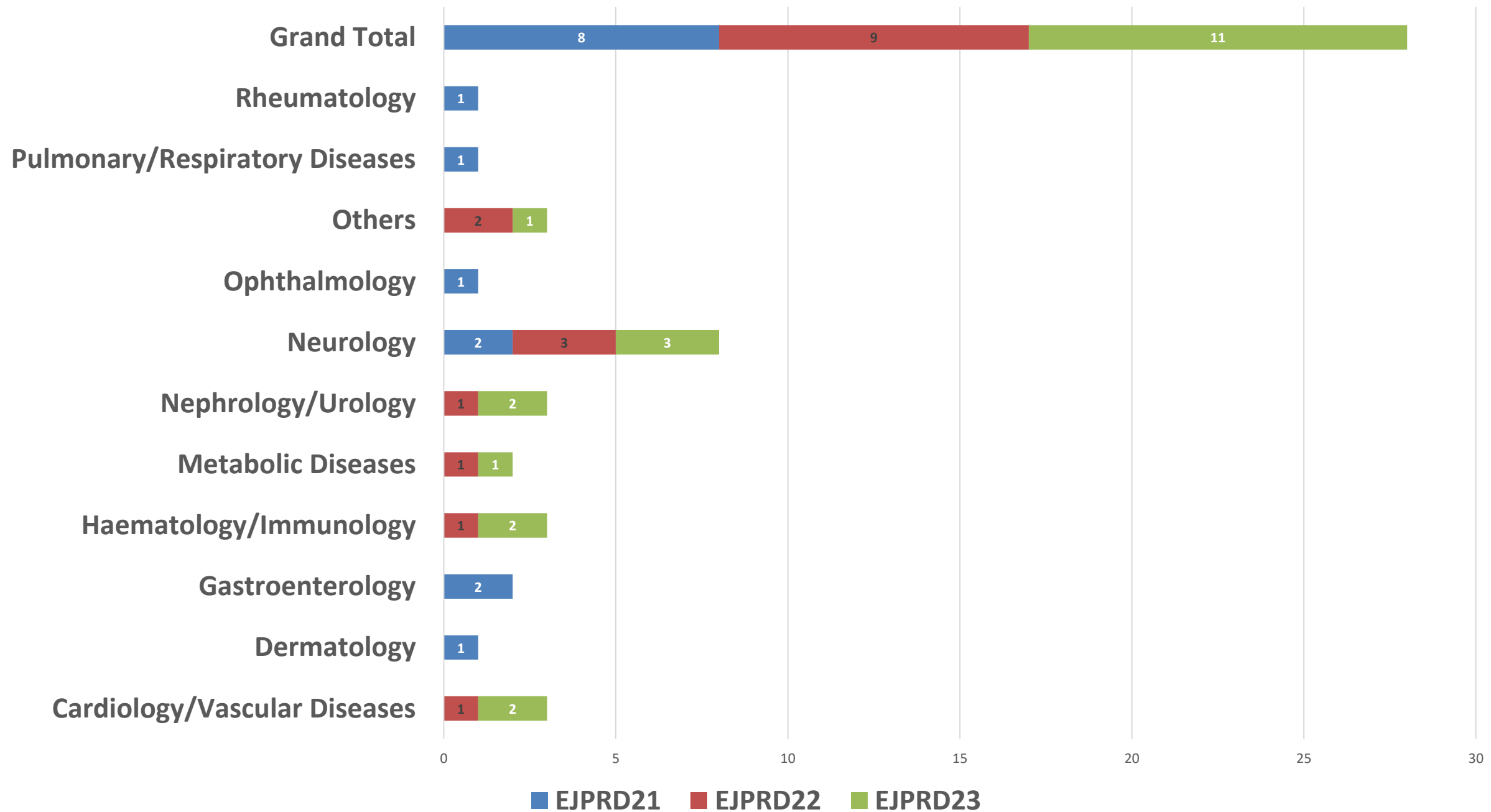
# Participation of ERNs in EJP RD joint transnational calls

Analysis of funded projects

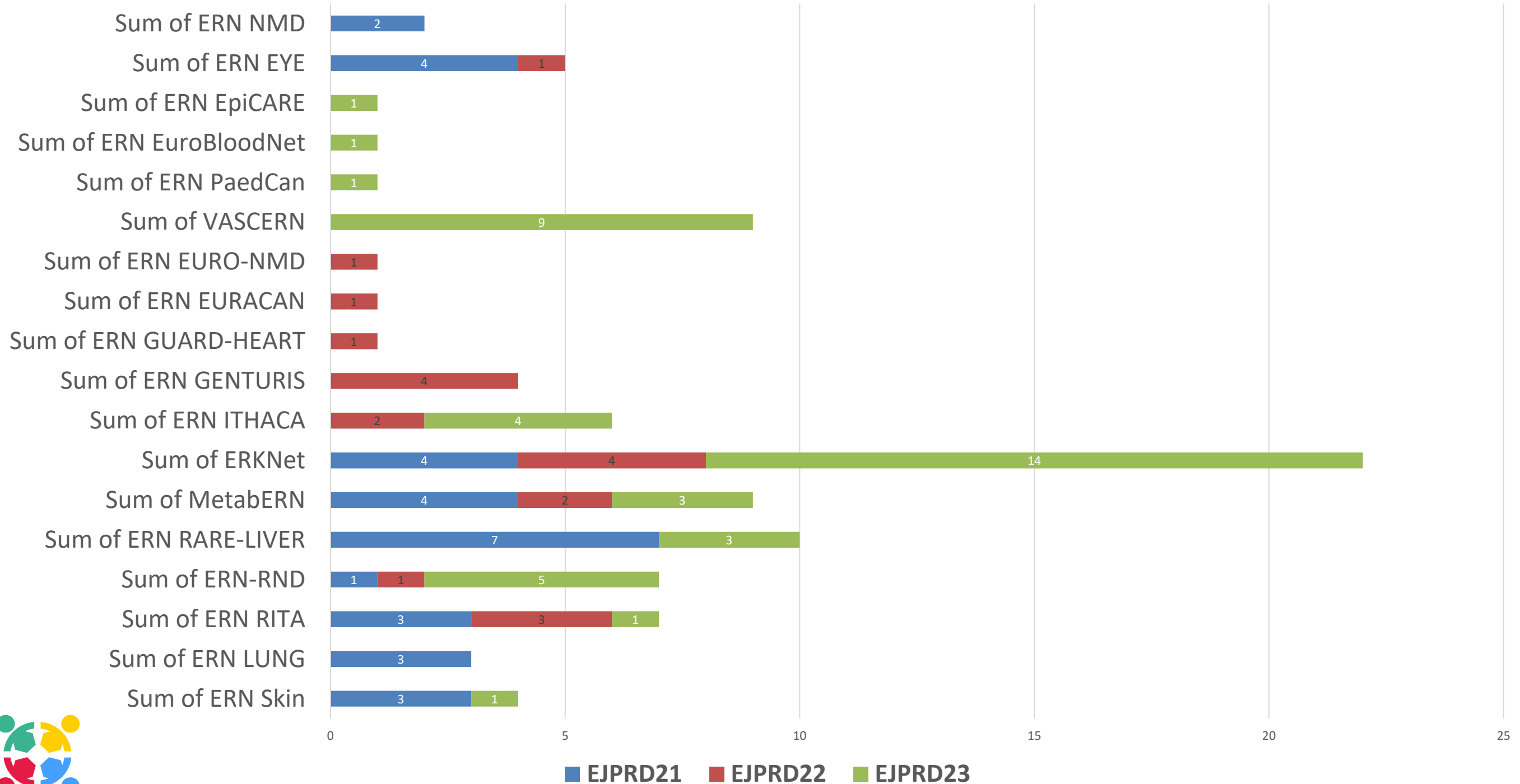
Funded projects with ERNs in JTC 2021-2022-2023  
(total of 28)



## Medical domains covered in JTC 2021-2022-2023 funded projects

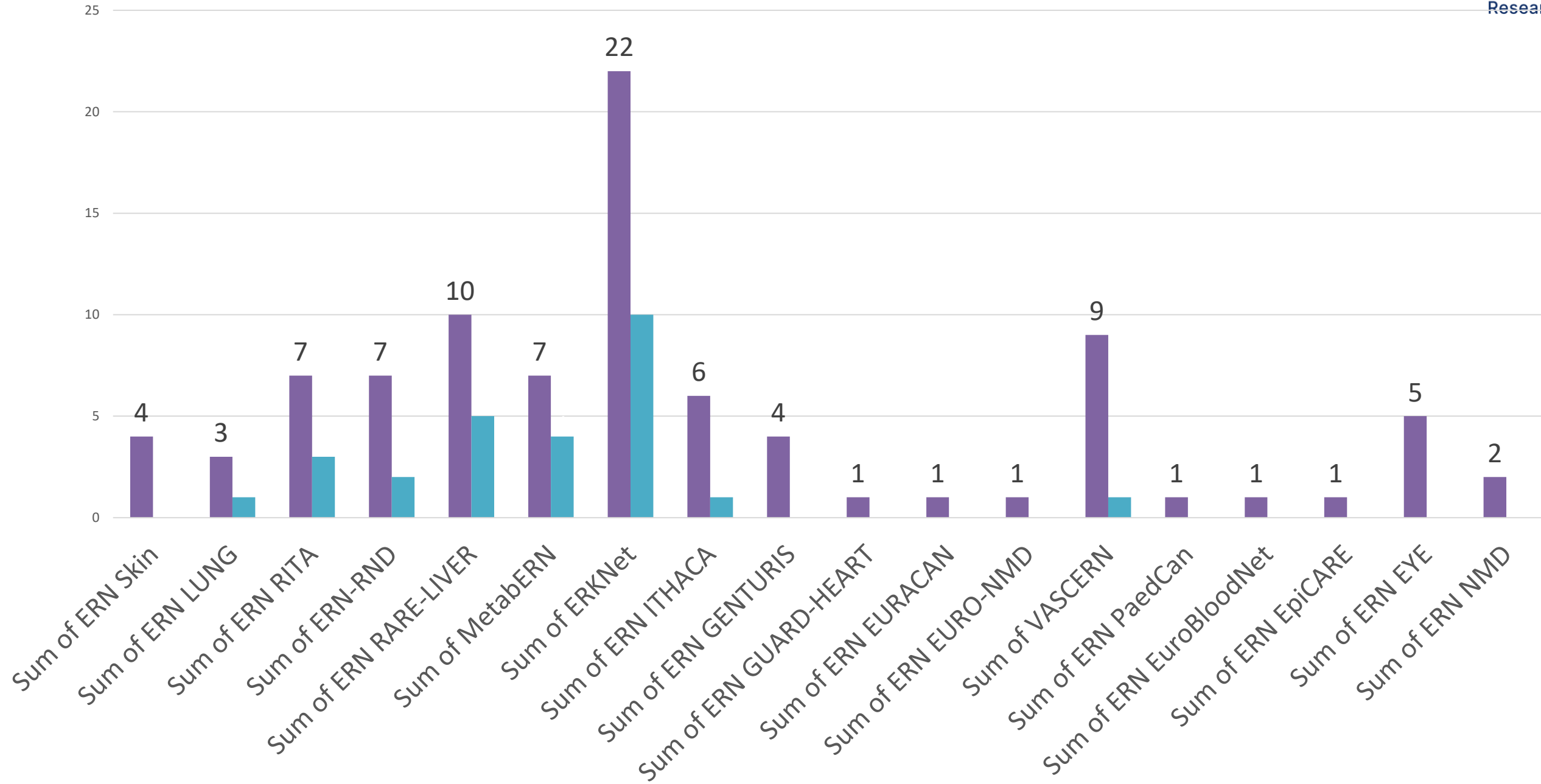


# Overall participation of ERNs in funded projects of EJP RD JTC 2021-2022-2023





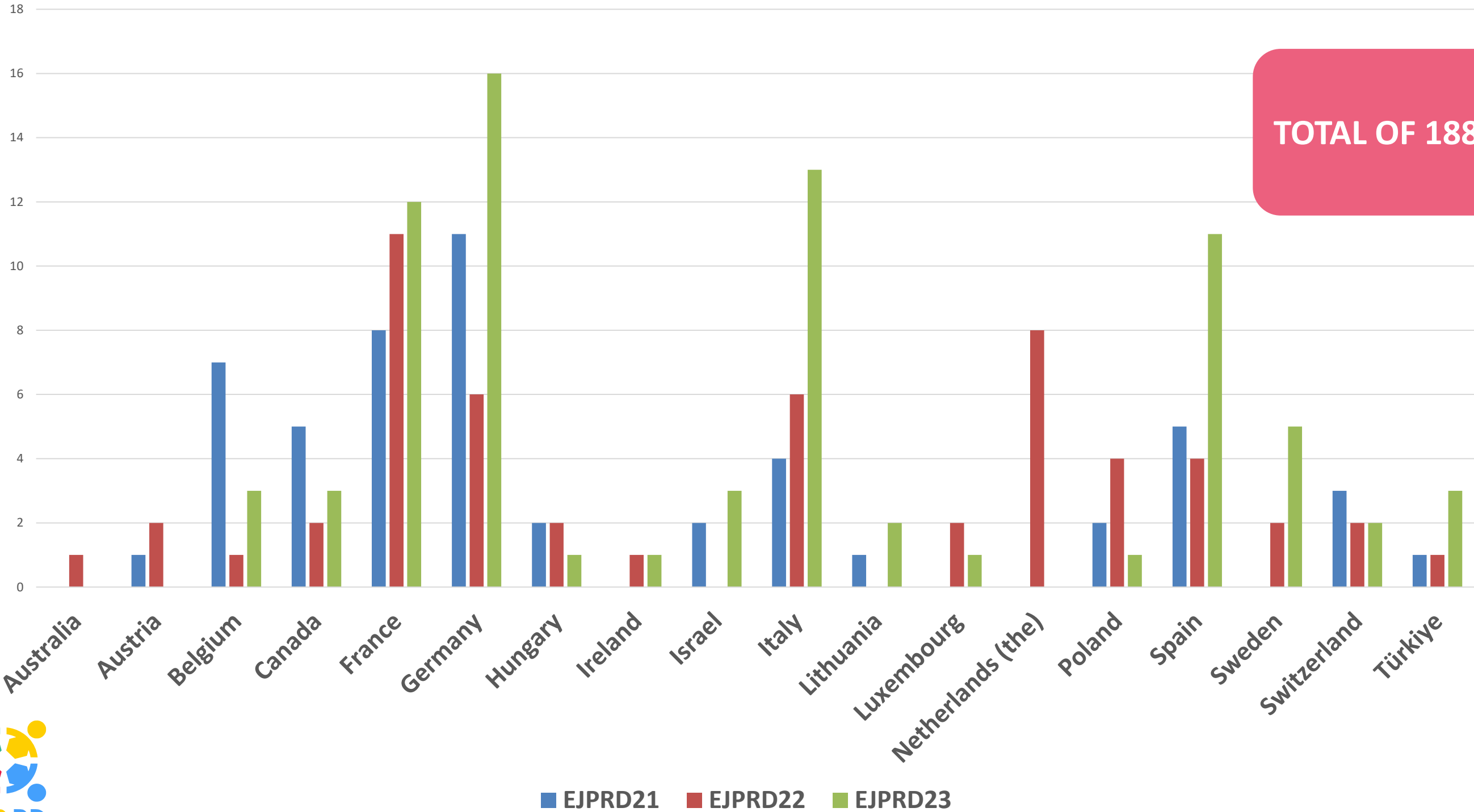
## Overall participation of ERNs in funded projects of EJP RD JTC 2021-2022-2023



■ Grand Total

■ Partners NOT confirmed by ERN HCP verification

# Countries in JTC 2021-2023-2023 funded project



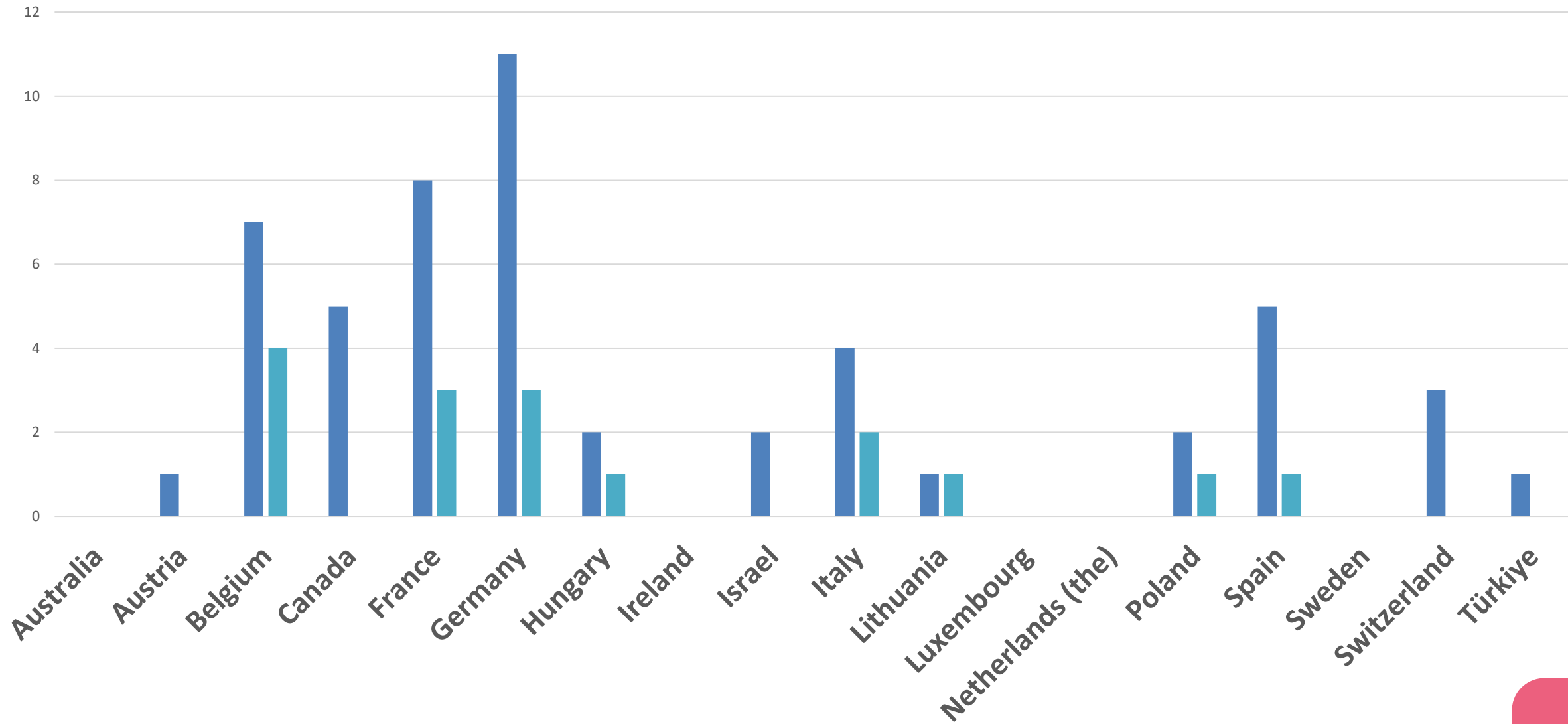
**TOTAL OF 188 TEAMS**



■ EJPRD21 ■ EJPRD22 ■ EJPRD23

## Participation of ERNs per country in JTC 2021

Social sciences and Humanities Research to improve health care implementation and everyday life of people living with a rare disease



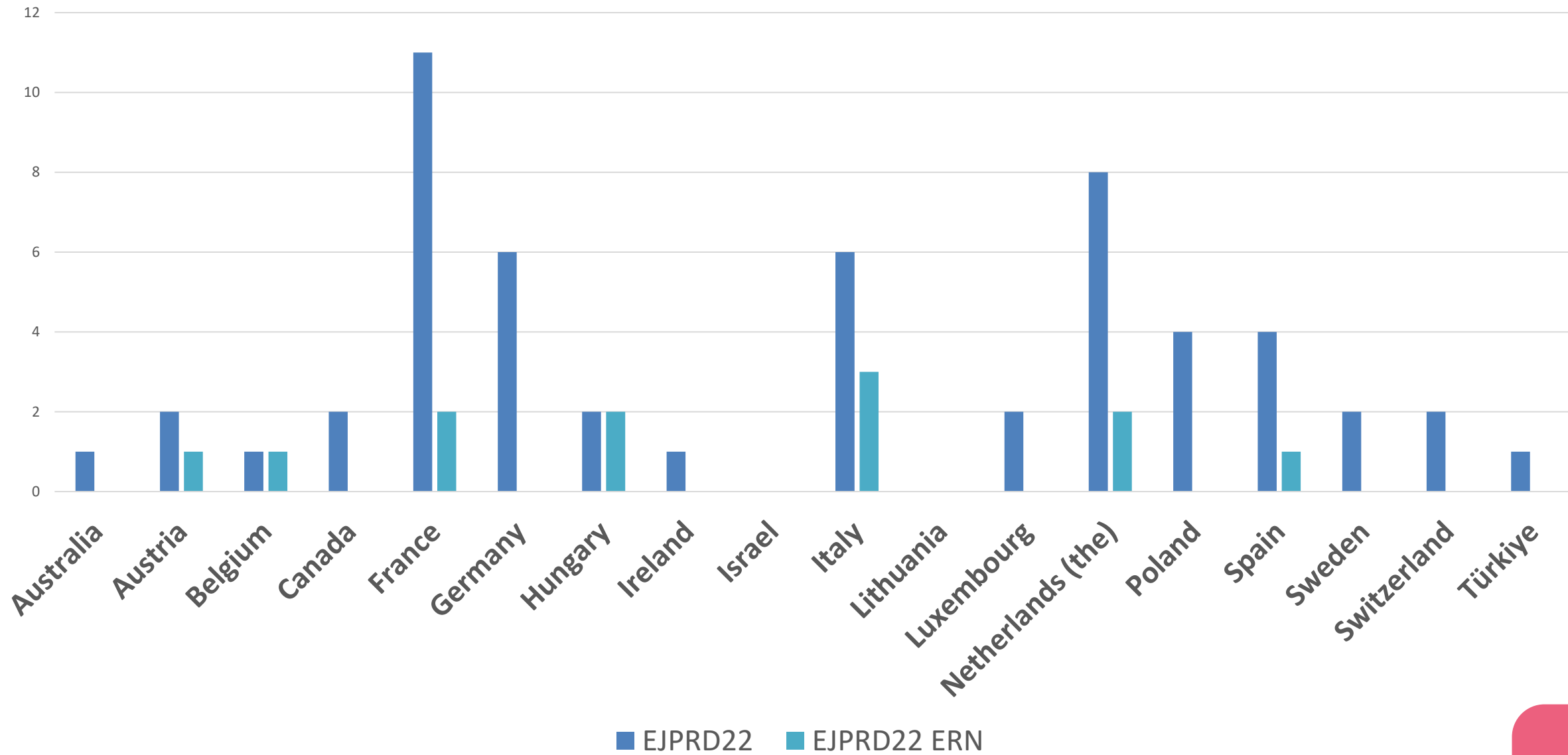
■ EJPRD21 ■ EJPRD21 ERN

**TOTAL OF 16 TEAMS**



## Participation of ERNs per country in JTC 2022

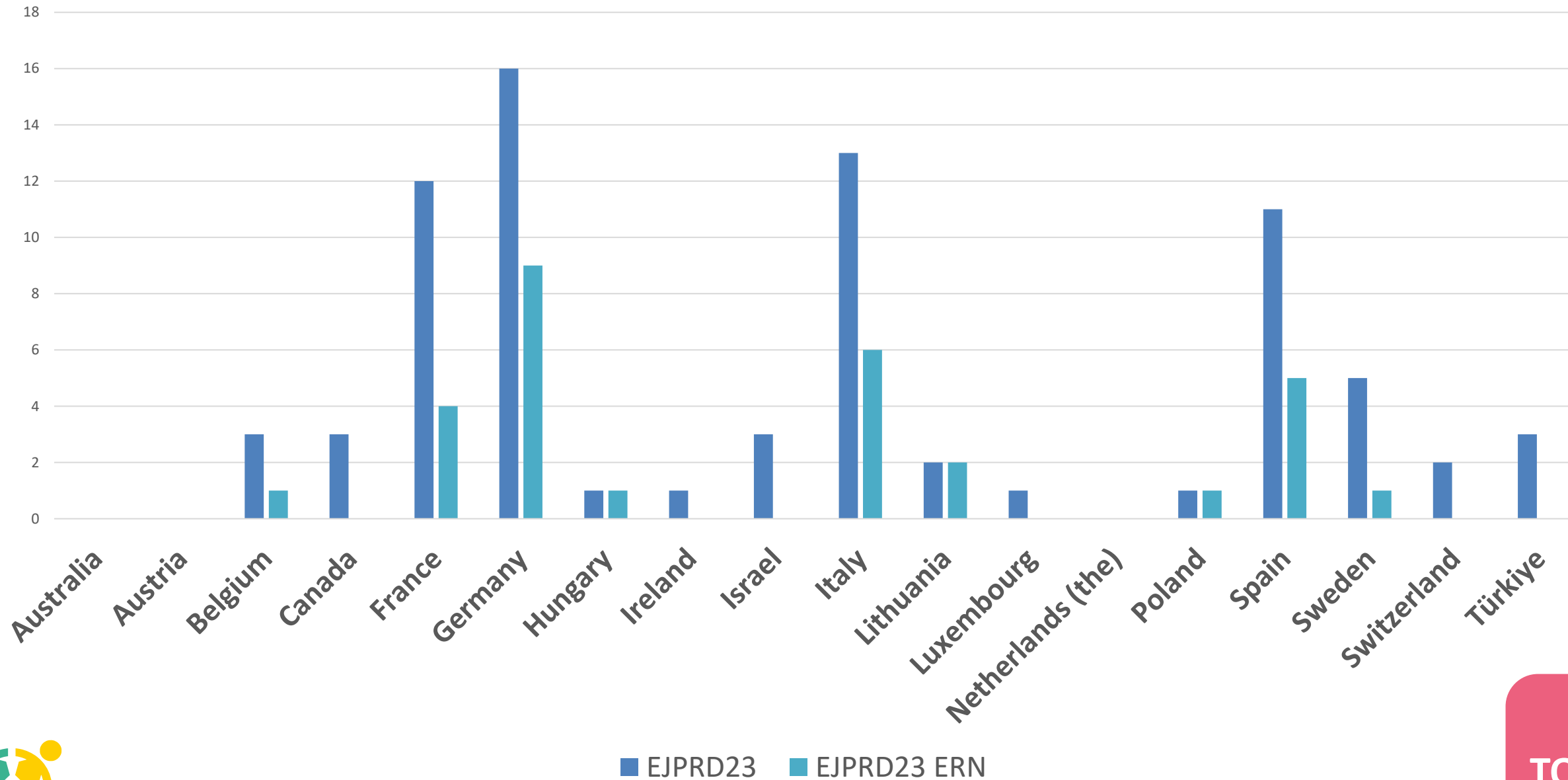
Development of new analytic tools and pathways to accelerate diagnosis and facilitate diagnostic monitoring of rare diseases



**TOTAL OF 12 TEAMS**

# Participation of ERNs per country in JTC 2023

## Natural History Studies addressing unmet needs in Rare Diseases



**TOTAL OF 30 TEAMS**



# ERN expertise brought in funded projects (analysis of CVs)

epidemiology  
epigenetics  
genetics evaluation  
computational healthcare  
molecular  
**clinical**  
genomics  
**research** biology  
bioinformatics translational  
medicine

# Expertise brought in funded projects by teams self-declared as ERNs (analysis of CVs)



# Expertise brought in funded projects by other teams than ERNs (analysis of CVs)

**cell-biology** **epigenetics** **pharmacoeconomics&QoL**  
**biostatistics** **economy/HTA** **microbiology** **biomarkers**  
**QoL&social-sciences** **signal-transmission** **biology** **immunopathophysiology**  
**metabolomics** **public-health** **omics&bioinformatics**  
**evaluation** **epidemiology&public-health**  
**translational-medicine** **clinical-genetics**  
**epidemiology** **proteomics** **social-sciences**  
**diagnostics** **genomics&bioinformatics** **sociology**  
**cell**  
**clinical-research**  
**genetics&bioinformatics** **developmental-biology** **medical-genetics**  
**genomics** **psychology&clinical-psychology**  
**psychology** **genetics&epigenetics** **epidemiology&economic**  
**molecular-biology**  
**biochemistry** **genetics** **psychology&healthcare**  
**pathophysiology** **animal-models(drosophila)**



# ERDERA

European **Rare Diseases**  
Research Alliance

## JOINT TRANSNATIONAL CALL 2025

ERICA ERN Research Conference  
11 – 13 of December 2024  
Udine, Italy



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.  
Views and opinions expressed are those of the author(s) only and do not necessarily reflect those of the European Union or any other granting authority, who cannot be held responsible for them.



# Call for Proposals 2025: Pre-clinical Therapy Studies for Rare Diseases

The ERDERA Call for Proposals 2025 is focusing on "Pre-clinical therapy studies for rare diseases using small molecules and biologicals – development and validation". This international collaborative initiative aims to advance research and development in the field of rare diseases, bringing together funding organizations from multiple countries to support innovative pre-clinical studies.

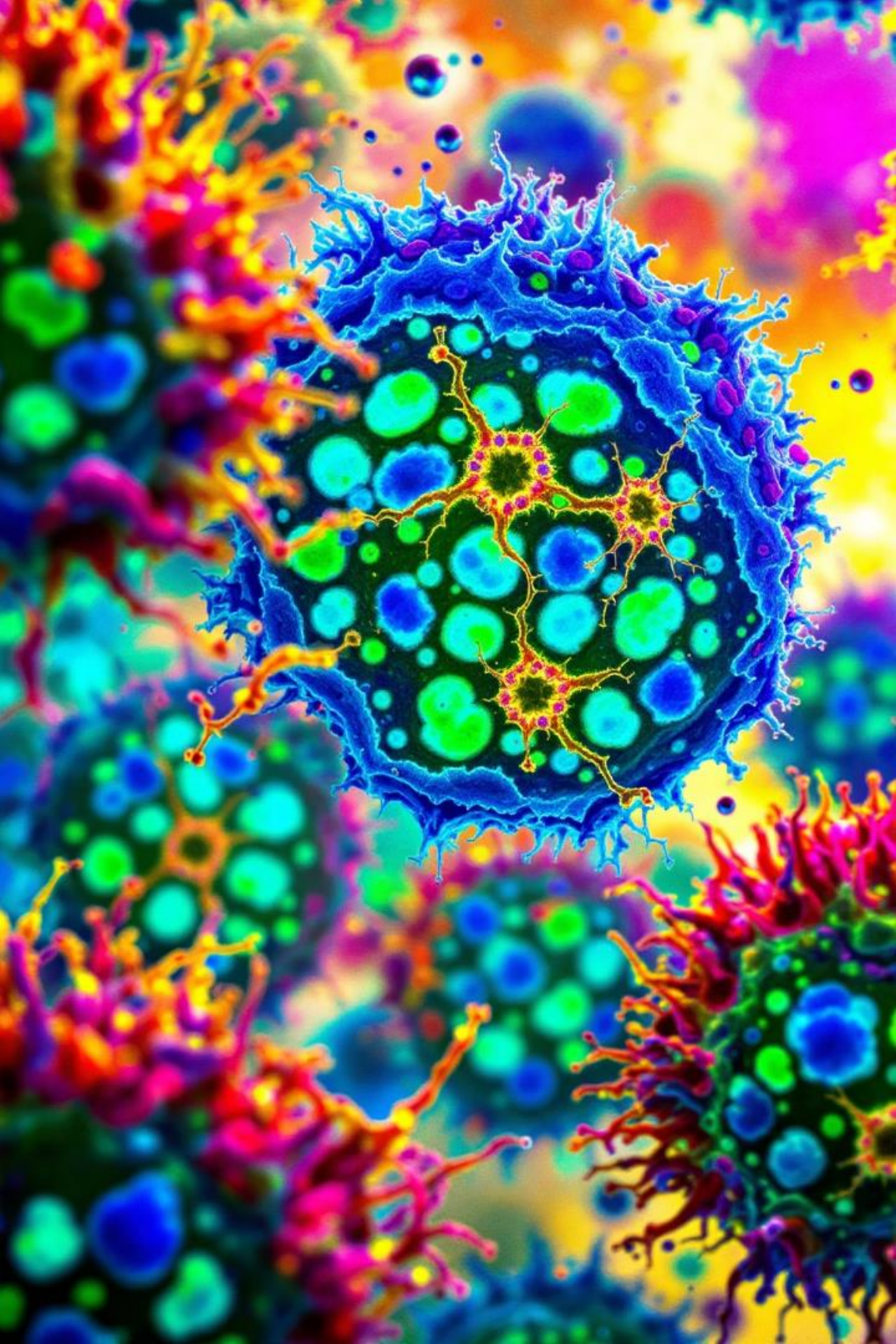
# Important Dates and Participating Countries



The call opens simultaneously with the involvement of funding organisations from 30 countries/regions, including **Austria, Belgium, Bulgaria, Canada, Cyprus, Czech Republic, Denmark, Estonia, France, Germany, Hungary, Iceland, Ireland, Israel, Italy, Latvia, Lithuania, Luxembourg, Norway, Poland, Portugal, Slovakia, Spain, Sweden, Switzerland, The Netherlands, and Türkiye.**

## Call Launch

- Call launch
- Form Consortium
- Build project
- Pre-proposal submission
  
- Invitation to second stage
- Full proposal writend stage writing, and evaluating, suamuation,
- Full proposal writing final decision



# Focus on Rare Diseases

## Definition

A rare disease affects not more than five in 10,000 persons in the European Community, EC associated states, and Canada.

## Scope

Projects should **focus on a group of rare diseases or a single rare disease if there is no valid rationale/evidence for the benefit of grouping diseases.**

## Commonalities

Applicants are strongly encouraged to work on groups of rare diseases with commonalities, based on state-of-the-art scientific discoveries and clinical practice.

# Call Topics and Research Areas

1

## Novel Therapies Development

Pre-clinical setting through cell, organoid and animal disease model studies, and/or use of in silico or artificial intelligence models to accelerate the success rate of the pre-clinical stage.

2

## Biomarker Development

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 Studies

Replication of pre-clinical studies in an independent lab to increase validity of exploratory findings.

4

## Pre-clinical Proof of Concept

Pre-clinical proof of concept studies for evidence of pharmacological activity *in vitro* and *in vivo*, pharmaco-kinetics and pharmaco-dynamics of the investigational drug (i.e., small molecule(s) and/or biologic) and first toxicology and safety data as well as studies to support readiness for initiating clinical trial authorization conforming to regulatory requirements.



# Excluded Approaches and Topics

## ATMP Therapies

Gene therapy medicinal product, somatic cell therapy medicinal product, tissue engineered product, according to EMA definition.

## Patient Registries

Set-up or extension of natural history studies / patient registries.

## Specific Neurodegenerative Diseases

Projects focusing only on rare neurodegenerative diseases 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.**

## Model Development

Development of new cell/organoid/animal models, which should already be established.

## Clinical Trials

Interventional clinical trials to prove efficacy of drugs/treatments/surgical procedures/medical procedures.

## Certain Disease Categories

Rare infectious diseases, rare cancers and rare adverse drug events in treatments of common diseases. **Rare diseases with a predisposition to cancer are eligible.**

# Consortium Requirements

## Partner Categories

- Academia
- Clinical/public health sector
- Enterprises (all sizes of private companies)
- Patient advocacy organisations (PAOs)

## Consortium Makeup

Each consortium must involve **four to six eligible principal investigator partners from at least four different participating countries**. One of these partners must be an Early Career Researcher (ECR). The number of partners can be increased to 8 in specific cases.

## Patient Involvement

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

They should clearly describe the role and responsibilities of the patient partners and PAOs in a patient involvement plan.

# Call Timeline





# Next Steps and Important Information

## 1 Review Call Details

Carefully read through all the call requirements, eligibility criteria, and research focus areas.

## 3 Engage Patient Partners

Reach out to patient advocacy organizations and include them in your proposal planning from the start.

## 5 Mark Key Dates

Note all important dates in the call timeline, especially the pre-proposal submission deadline of February 13, 2025.

## 2 Form Your Consortium

Identify potential partners from different participating countries, ensuring you meet the consortium makeup requirements.

## 4 Prepare Your Pre-proposal

Begin drafting your pre-proposal, ensuring it aligns with the call's objectives and requirements.

## 6 Seek Additional Information

Visit <https://erdera.org/funding/> for more detailed information and updates on the call.

# ERD ERA

European Rare Diseases  
Research Alliance

[Visit our website](#)



# Thank you!

Follow us on **social media**

---



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.

Views and opinions expressed are those of the author(s) only and do not necessarily reflect those of the European Union or any other granting authority, who cannot be held responsible for them.