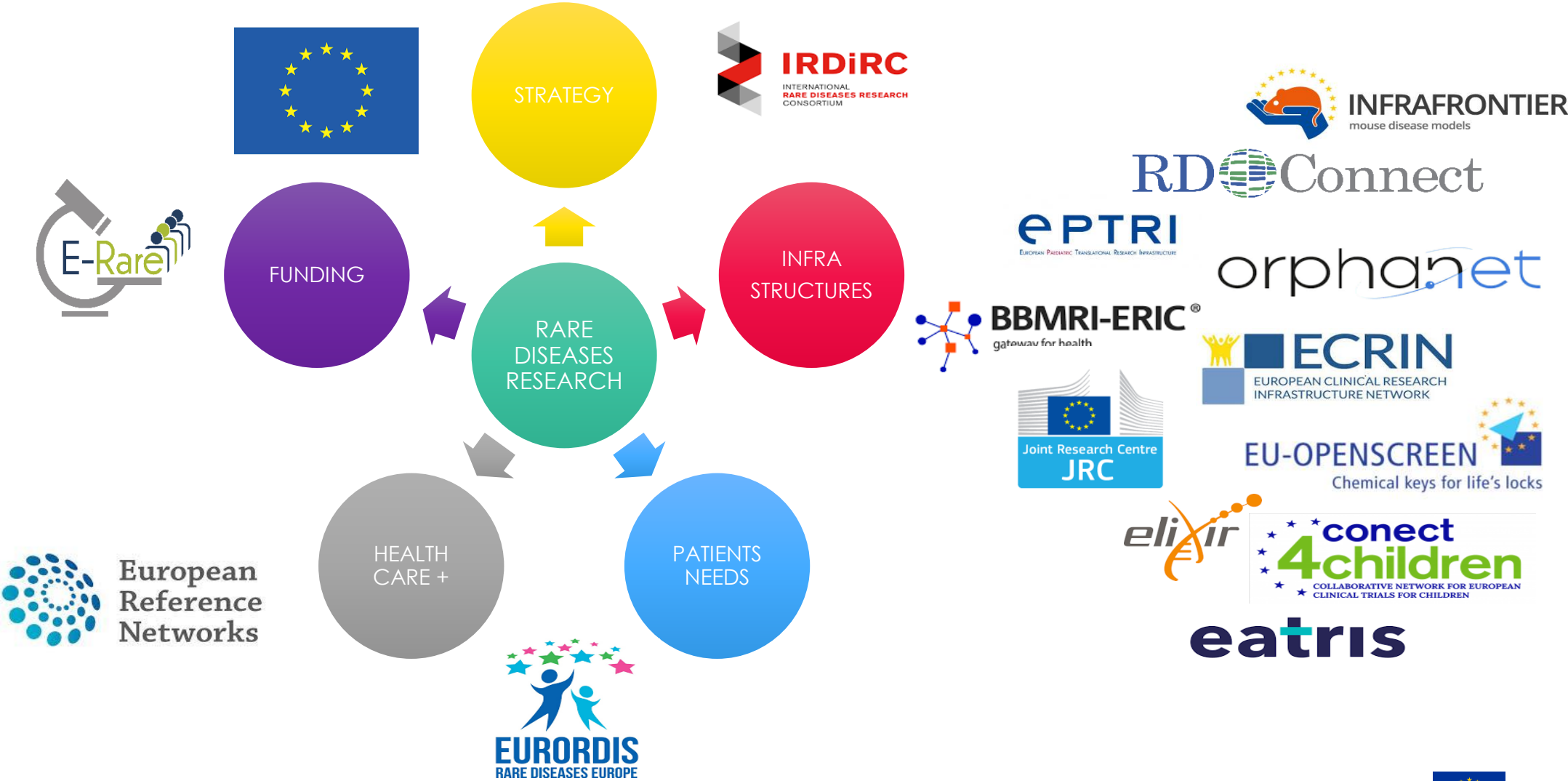


# European Joint Programme Cofund on Rare Diseases

Daria Julkowska  
INSERM, France

TO REACH Workshop « Funding a structured approach to  
European health services and systems research »  
11 of April 2019, Brussels, Belgium

# Rare Diseases Landscape in Europe



# Why EJP Cofund?

Features	ERA-Net Cofund	EJP Cofund	Art.185 Initiative
<b>Level of integration</b>	Low	High	High
<b>Who can participate</b>	Only POs and PMs with capacity to finance research through open calls	PO, PM, RPOs (if they are mandated by PO), other relevant partners	Participating states
<b>Who signs</b>	For MS: PO and PM (RPOs exceptionally under in kind ERA-Net scheme) For EU: European Commission	For MS: PO and PMs (especially RPOs), other type of legal entities For EU: European Commission	Decision of Parliament and Council Delegation agreement is signed between Commission and DIS (Designated implementation Structure)
<b>Open joint calls</b>	Yes	Yes	Yes
<b>Central grant management</b>	Not necessary but possible	Not necessary but possible	Yes (no involvement of funding agencies expected)
<b>Direct research activities</b>	No with exception of in kind ERA-Net	Yes	Yes
<b>Annual work plan</b>	No	Yes	Yes
<b>Type of actions possible to include</b>	Funding + additional activities (direct research activities limited to in kind ERA-Net)	Funding + research activities + mobility & training + coordination actions	Funding + research activities + mobility & training + coordination actions
<b>Reimbursement rate</b>	Max. 33%	Negotiable (50 - 70%)	Max. 50% COM contribution to costs of the programme

# 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

# Main facts about the EJP RD

Jan 2019

Dec 2023

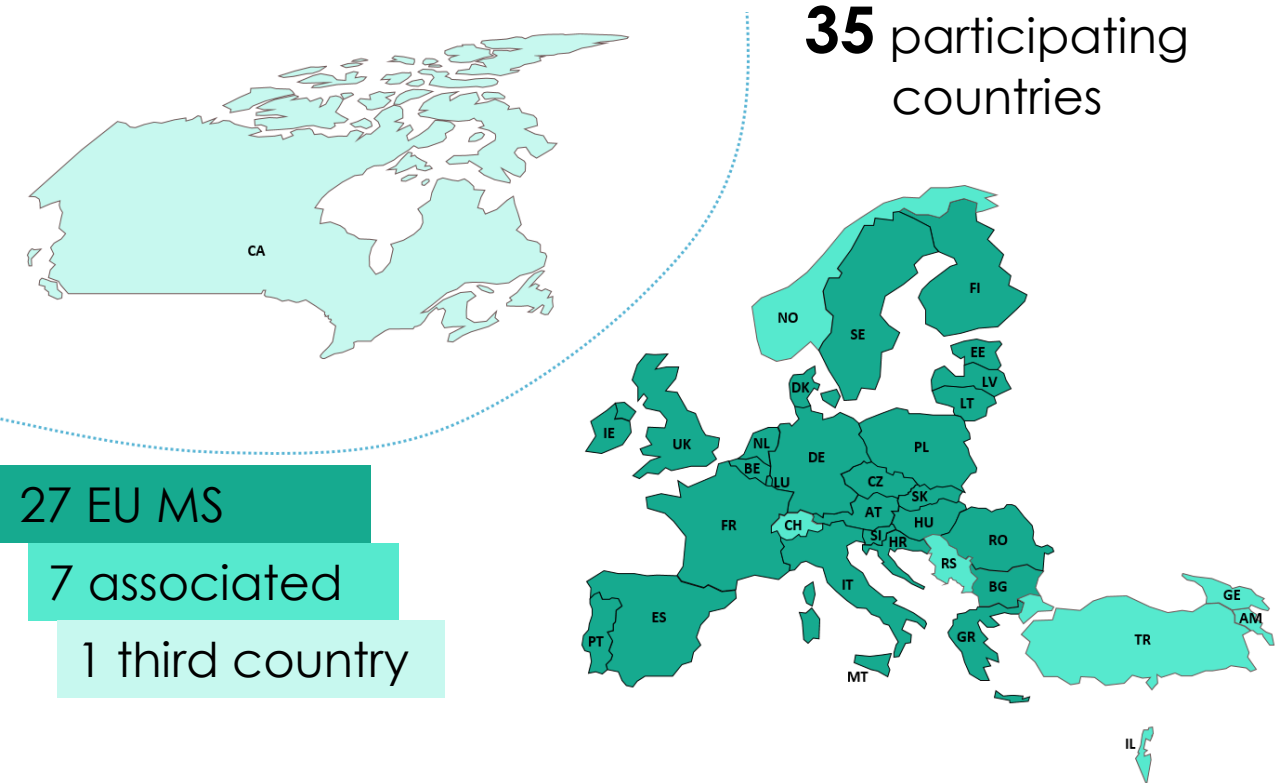
**Total budget** (min. submitted): **101 M€** (→ expected > 110 M€)

**Union contribution: 55 M€** (70% reimbursement rate)

**35** participating countries

**88** beneficiaries

- 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)
- + 50 Linked Third Parties



# EJP RD STRUCTURE

Coordinated by





# WP1 COORDINATION & MANAGEMENT

WP2  
STRATEGY

WP3  
SUSTAINABILITY

WP4  
ETHICS, LEGAL, REGULATORY & IPR

WP5  
COMMUNICATION & DISSEMINATION



WP6  
Joint Transnational Calls

WP7  
Networking scheme

WP8  
RDR Challenges

WP9  
Monitoring of funded projects



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



WP 14  
Training on data management & quality

WP 15  
Capacity building and training of patients and researchers

WP 16  
Online Academic education course

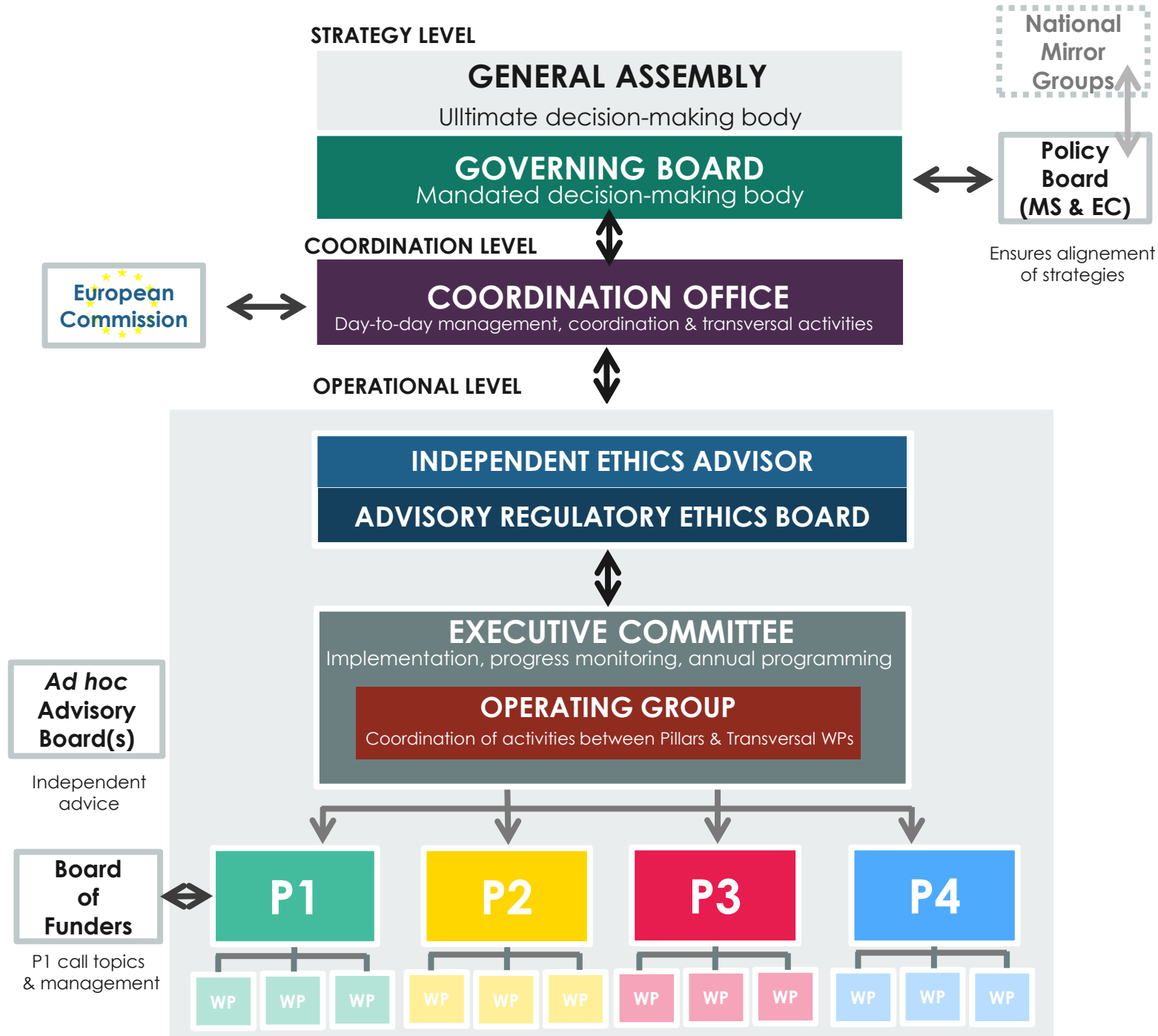
WP 17  
ERN RD training and support programme

WP 18  
Development and adaptation of training activities



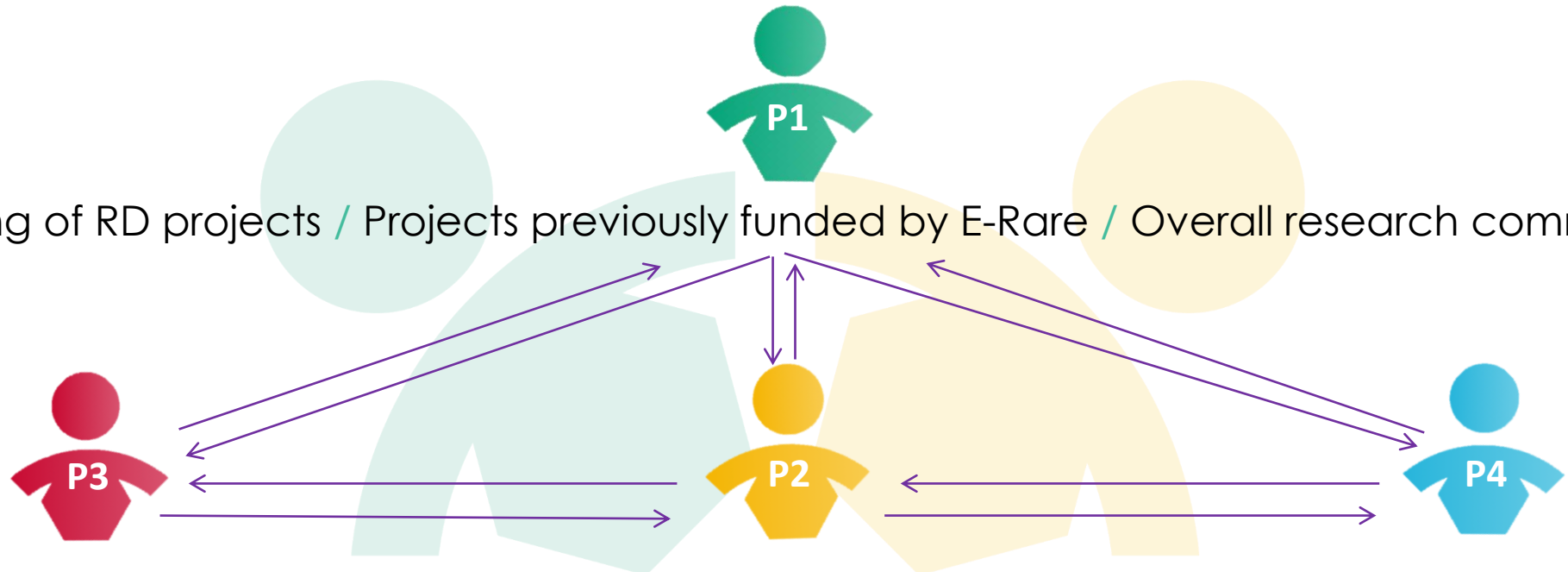
WP 19  
Facilitating partnerships and accelerating translation

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





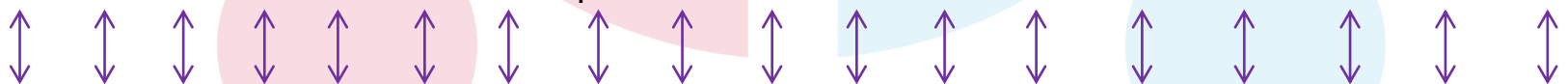
Funding of RD projects / Projects previously funded by E-Rare / Overall research community



- Access to dedicated trainings
- Increased knowledge of new generations
- Development of new relevant trainings

- Access to and deposit of data
- Availability of additional resources & tools
- Contribution to the development of the virtual platform, interaction and input for ERNs

- Access to direct support by innovation managers & tools
- Direct expertise from ERNs
- Translation of gen(omic) results into accelerated diagnosis & treatment



**INTERNATIONAL, EU, NATIONAL, REGIONAL STRATEGIES & FACILITIES**



# Main challenges vs main advantages

## Main challenges:

- ⌘ Requires long-term preparation & negotiation → commitment of institutions & people is crucial (also financial for the preparation phase)
- ⌘ If really considered as “one body” finding an agreement among diverse participating parties may be challenging
- ⌘ Heavy administrative burden → specialised coordination team is needed
- ⌘ Possible “sophisticated” financial arrangement → depending on the choice of the financial approach
- ⌘ The problem of beneficiary vs third party status for open calls for projects

## Main advantages:

- ⌘ High level of flexibility → different type of activities are possible (with different reimbursement rates if agreed by the consortium)
- ⌘ Reimbursement rate up to 70%
- ⌘ Annual flexibility allowing better response and alignment with strategic objectives at national & EU level
- ⌘ True commitment = true recognition & higher weight in policy/strategic negotiations (including sustainability)
- ⌘ Increased efficiency, decreased overlaps & optimisation of budgets in the domain
- ⌘ High impact for the community

# THANK YOU

[www.ejprarediseases.org](http://www.ejprarediseases.org)

[daria.julkowska@inserm.fr](mailto:daria.julkowska@inserm.fr)

[coordination@ejprarediseases.org](mailto:coordination@ejprarediseases.org)