

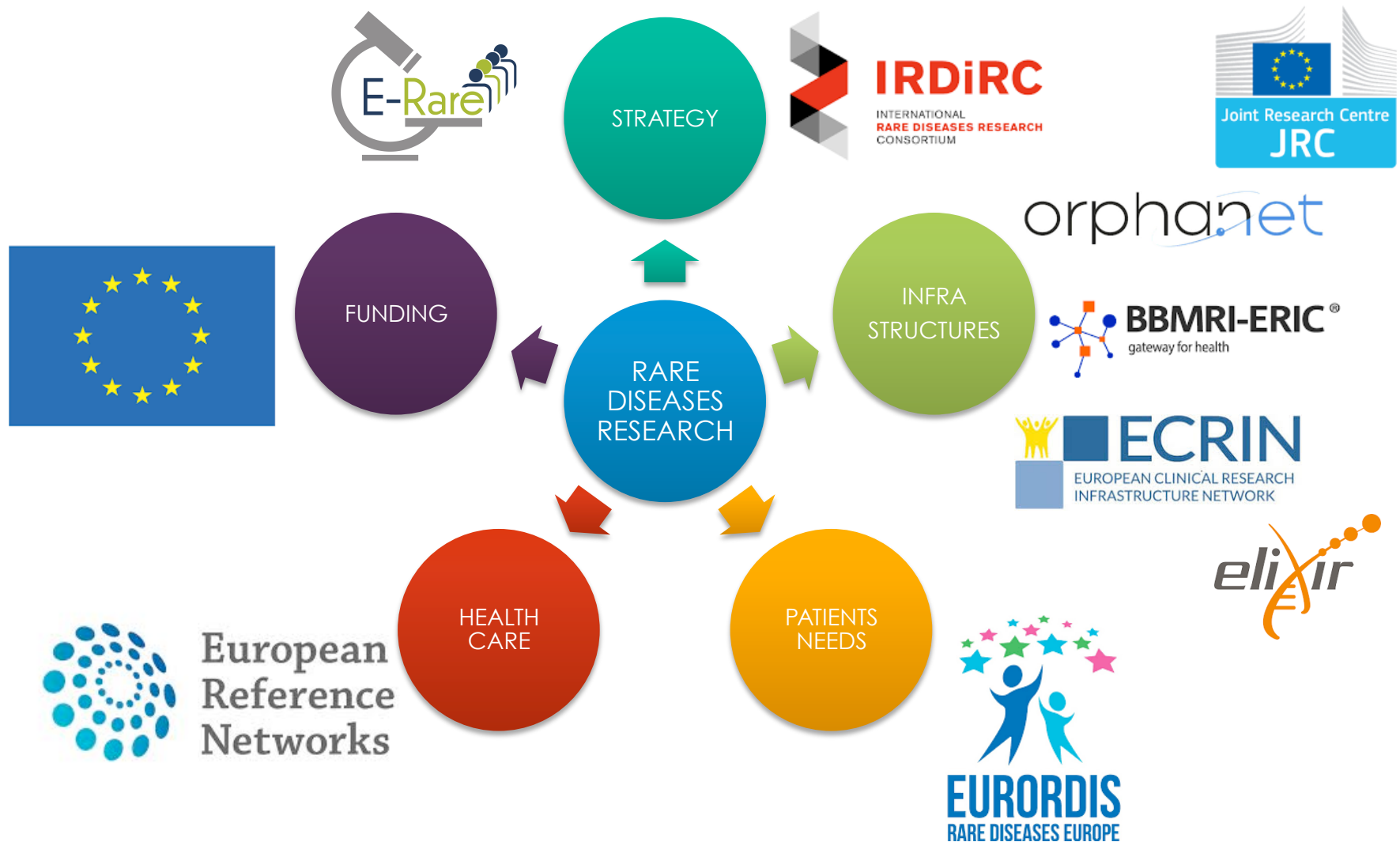
# LA FRANCE DANS LA RECHERCHE EUROPÉENNE ET INTERNATIONALE SUR LES MALADIES RARES

- RÉUSSITES ET PERSPECTIVES -

DARIA JULKOWSKA

RARE 2017 – LES 5<sup>EME</sup> RENCONTRES DES MALADIES RARES  
PARIS 20-21 NOVEMBRE 2017

# RARE DISEASES LANDSCAPE IN EUROPE



# PLACE OF FRANCE IN THE EUROPEAN RARE DISEASES LANDSCAPE

- France is involved in the International Rare Diseases Research Consortium from the start (2011), represented by ANR, FFRD & AFM and actively contributes to its goals. The Scientific Secretariat of IRDiRC is under the responsibility of INSERM
- France is part of all European Reference Networks and coordinates 5 of them
- France is involved in all EU medical infrastructures (BBMRI, EATRIS, ECRIN, Infrafrontier, EU-Openscreen, Elixir...) and hosts central office of ECRIN
- Orphanet is hosted and was created in France
- France hosts offices of EURORDIS
- France coordinates E-Rare ERA-Net from its inception

# LA FRANCE ET E-RARE

# FUNDING OF RARE DISEASES RESEARCH IN FRANCE

- There is no specific public rare diseases research funding programme in France
- ANR provides public funding for rare diseases through its “generic” calls, which represents 8 – 15 M€/year = 10% of biology & health projects and funding
- Rare diseases research is also supported via PIA (Plan d'Investissements d'Avenir) which supports for example RaDiCo, IMAGINE or F-CRIN and corresponds to 15 – 20 M€/year
- DGOS supports rare diseases by direct funding of rare diseases reference centers and networks as well as through open calls for clinical research (PHRC programme), which equals about 20 M€/year for care and research
- Funding for rare diseases is also ensured by the French Foundation for Rare Diseases and AFM Telethon

# MAJOR FACTS ABOUT E-RARE

- ERA-NET for research programs on rare diseases
- Created in 2006 (E-Rare-1) renewed in 2010 (E-Rare-2) at present funded under Horizon 2020 ERA-Net Co-fund

- E-Rare-3 (2014 - 2019)



- Coordinated by INSERM (2006 – 2014) and ANR (2014 – 2019)
- 28 partners from 20 European, Associated and non-European countries (AT, BE, CA, CZ, CH, ES, FR, GR, HU, IT, IL, JP, LT, LV, NL, PO, PL, RO, TR)
- **Objective: coordinate and promote European and international efforts for funding research on rare diseases**
- E-Rare is mentioned in more than half of National Plans for Rare Diseases (including French)



# E-RARE ACTIVITIES

## Coordination & Support

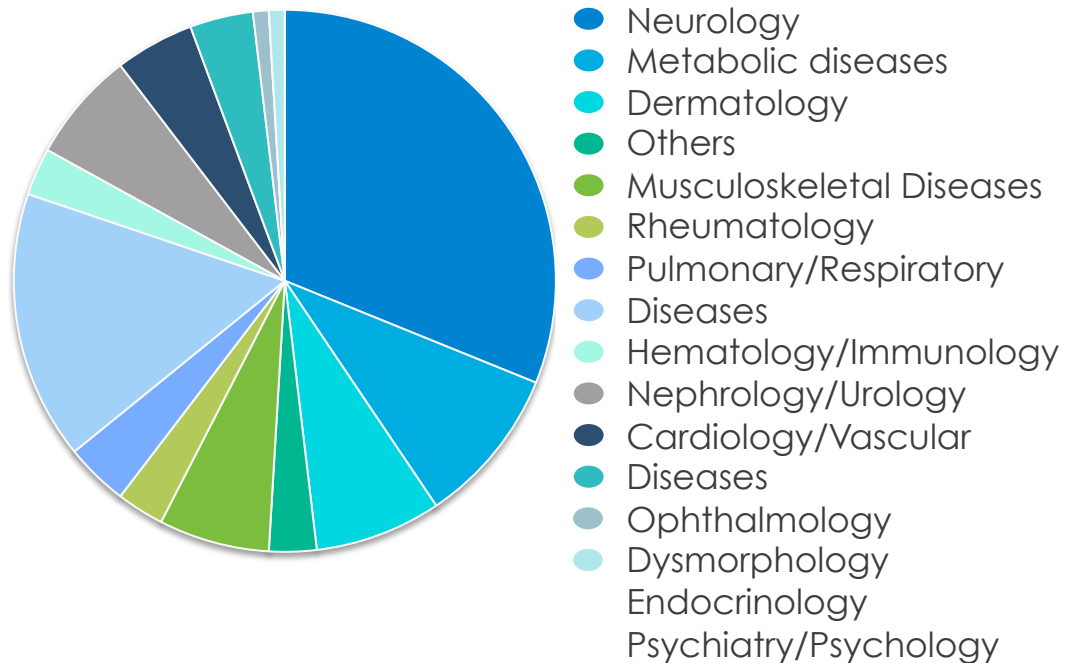
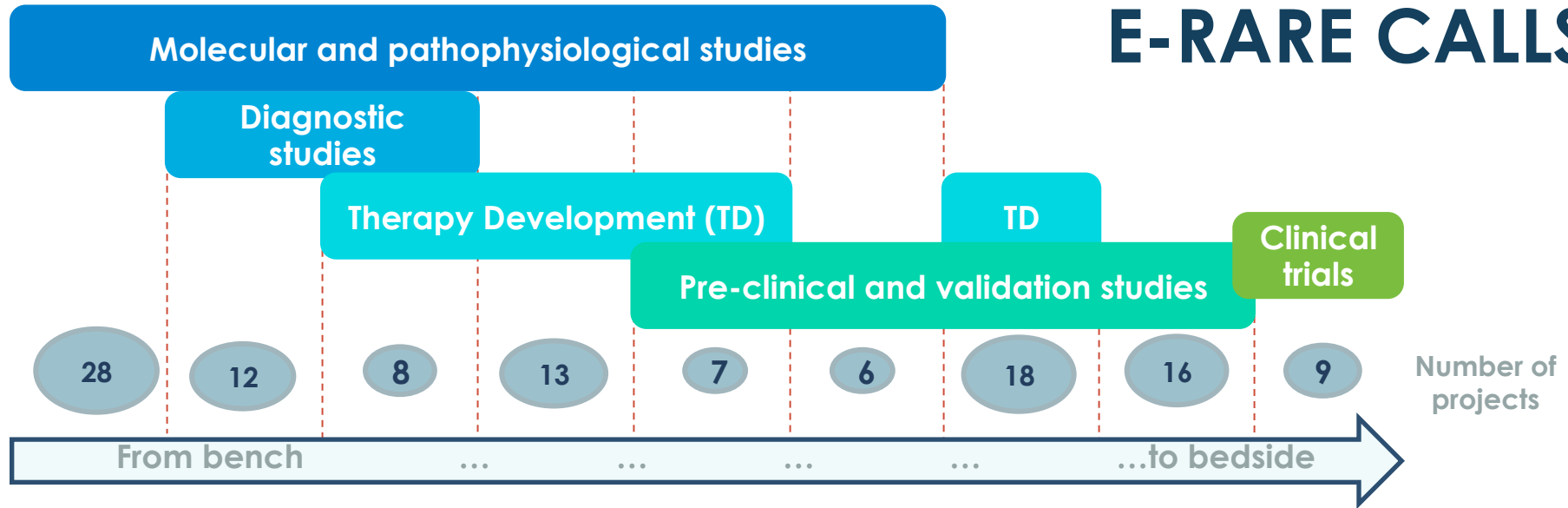
- Harmonization of national programmes
- Development of strategic agenda & active contribution to IRDiRC
- Development of collaborations with relevant stakeholders: EU Research Infrastructures, EURORDIS, EMA, etc.

## Joint Transnational Calls

- Launched yearly
- Based on Member States (MS) & EC contributions (70%-30%)
- Variable participation of MS  
Topics defined according to the strategic priorities and needs of the RD community: cover from large RD focus to specific calls on repurposing in CTs or novel therapeutic approaches



# E-RARE CALLS



Medical domains represented in the funded projects

**9** Joint Transnational Calls:  
**117** funded projects  
**104 M€** invested  
 Including **556** research partners funded



# FRANCE IN E-RARE CALLS

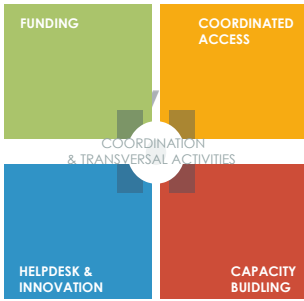
- France participates in 90% of proposals
- The success rate of French teams is higher than average: **33.3%** vs 25% (2<sup>nd</sup> stage) and **11.6%** vs 10% (global)
- Out of 117 funded projects **89 include FR research teams**
- A total of **119 FR research teams were financed** within 9 E-Rare calls
- Within 9 calls ANR invested **15.32 M€** and DGOS **1.14 M€** (JTC 2016) and patient organisations **0.3 M€**, in addition FR teams profited from **1.1 M€** of EC co-funding (JTC 2015)
- The leverage effect of projects with FR teams is of 1 to 6 → for every 1€ invested by France other countries invested 6€ in the same project!

# E-RARE JOINT TRANSNATIONAL CALL 2018

**TOPIC: Transnational research projects on hypothesis-driven use of multi-omic integrated approaches for discovery of disease causes and/or functional validation in the context of rare diseases**

- With participation of 18 countries: AT, BE, CA, CZ, CH, DE, ES, FI, FR, GR, HU, IL, IT, LV, NL, PL, RO, TK
- Tentative budget above 10 M€
- Call opens on 7 December 2017 → pre-proposal submission deadline 6 of Feb 2018
- Focus on collaborative projects on rare diseases (min. 3 teams from 3 different countries)
- Excluded: rare cancers, rare infectious diseases, rare adverse drug events/medical complications in treatments of common diseases & interventional clinical trials
- Foreseen ANR budget 1.5 M€
- MORE INFO AT: **[www.erare.eu](http://www.erare.eu)**

**LA FRANCE ET  
EUROPEAN JOINT PROGRAMME  
ON RARE DISEASES (EJP RD)**



# EUROPEAN JOINT PROGRAMME ON RARE DISEASES

## OBJECTIVES

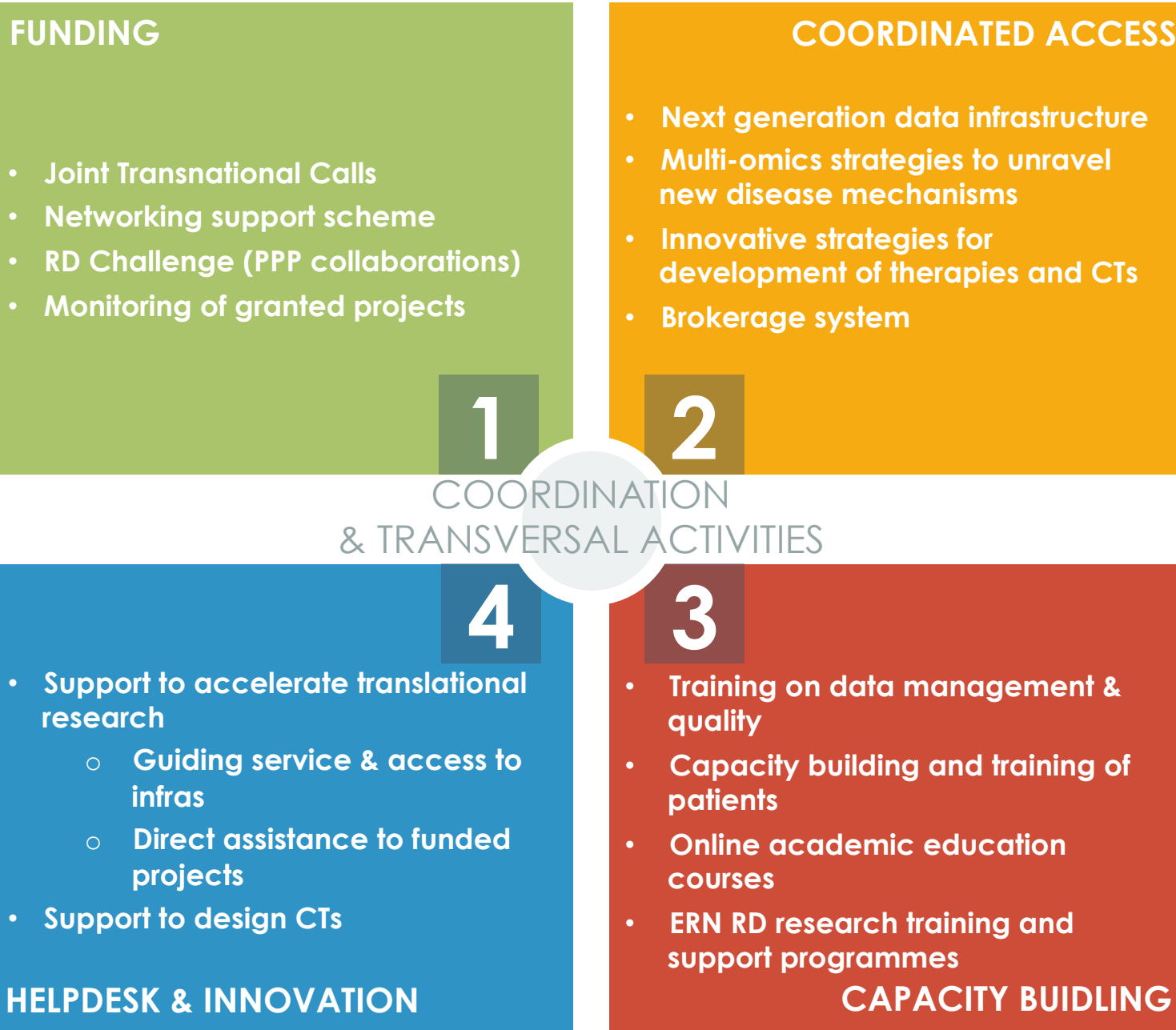
- **Main objective:** Create a research and innovation pipeline "from bench to bedside" ensuring rapid translation of research results into clinical applications and uptake in healthcare for the benefit of patients
  - **Specific objective:** improve integration, efficacy, production and social impact 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
- 
- **Union contribution:** 55 M€
  - **Total budget (min):** 78,5 M€ (→ expected > 110 M€)
  - **Foreseen number of partners:** 70 – 100
  - **Foreseen number of participating countries:** 29 including 23 EU MS, 4 associated (CH, IL, NO, TK) and third countries (AU, CA, JP, USA?)

# LEADING ROLE OF FRANCE

## Background of preparatory work

- **March 2016 - First discussions started at E-Rare strategic workshop in Barcelona**
- August 2016 - The Commission sends official invitation to MS to nominate experts to the EJP RD working groups
- **October 2016 - First meeting of the MS EJP RD expert group – France (MESRI, INSERM & E-RARE) takes the lead**
- Dec 2016 – Feb 2017 - Preparation of the 1<sup>st</sup> Concept Draft of the EJP RD
- **April 2017 - 2<sup>nd</sup> meeting of the MS EJP RD experts group – organization in Pillars**
- Apr – June 2017 – nomination of new experts to future WGs
- **June 2017 – kick off meeting of the extended WGs of the EJP RD: INSERM, Orphanet, IMAGINE and EURORDIS lead WGs**
- June – Oct 2017 – preparation of the 2<sup>nd</sup> Concept Draft of the EJP RD → more than 70 experts involved + 24 ERN coordinators representing EU, associated and non-EU countries → **15 experts from FR**
- **12 Oct 2017 – 2<sup>nd</sup> EJP RD Concept Draft sent to the EC and WGs + ERN Coos**
- **17 Oct 2017 – last meeting of the MS**

# EJP RD STRUCTURE





# TRANSVERSAL ACTIVITIES

## STRATEGIC COORDINATION & MANAGEMENT

- Effective operational management to reach objectives
- Procedures & tools for timely delivery and progress
- Administrative organization & follow up
- Strategic steering (alignment at regional/national/EU/international level)
- Scientific programming (call topics, yearly work plans, **Scientific Secretariat of IRDiRC**)

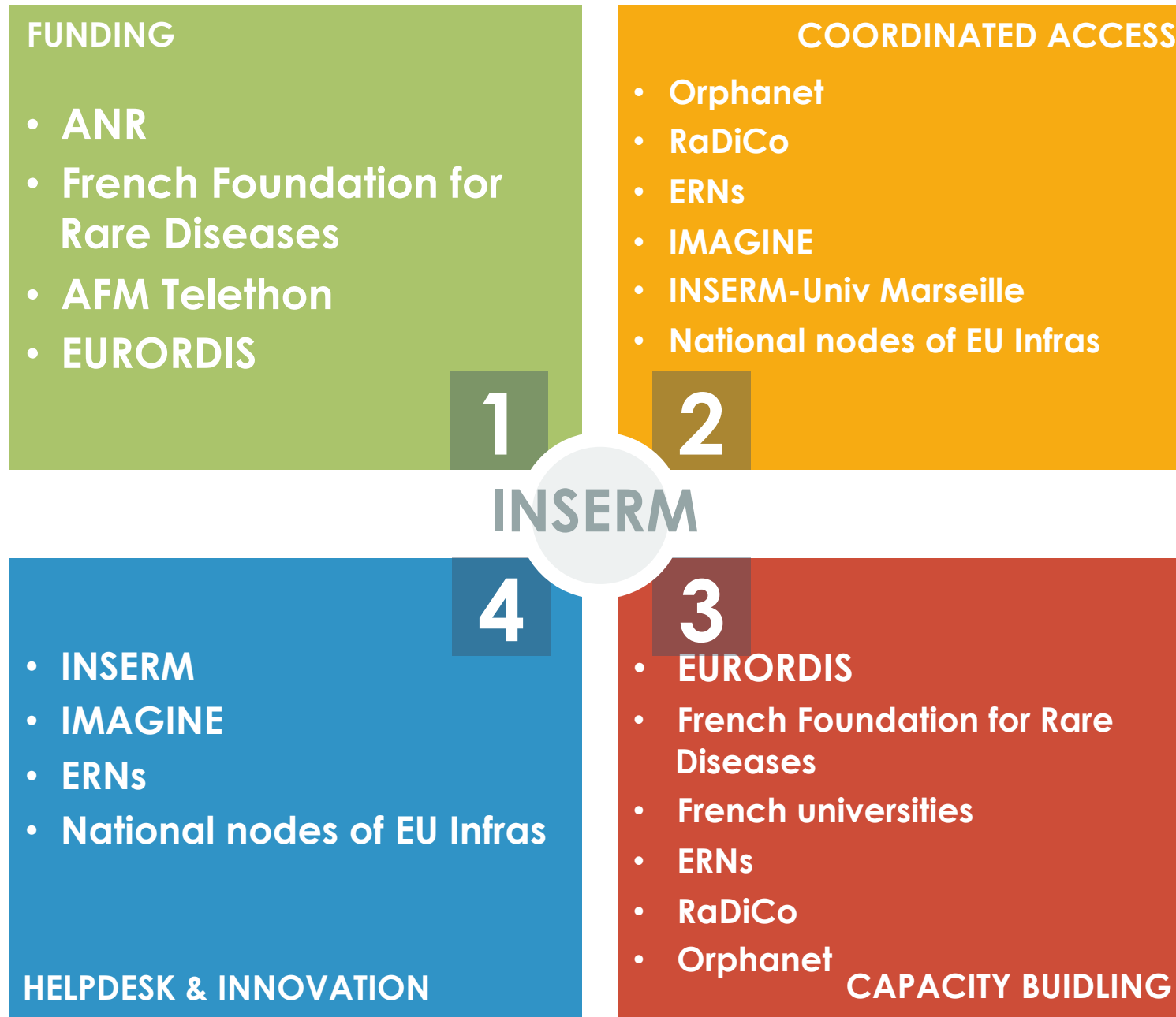
## COMMUNICATION & DISSEMINATION

- Internal communication tools
- External communication tailored to different types of stakeholders
- Dissemination of EJP RD results (promotion of EJP RD structures, products and tools; promotion of results of funded projects)
- Support to regional/national communication of involved stakeholders

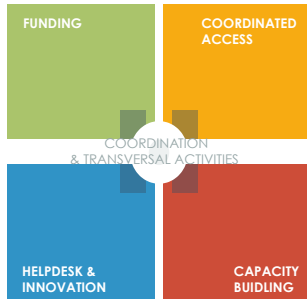
## SUSTAINABILITY

- Long-term vision and business model for the EJP RD as a whole and for specific activities & products

# EJP RD STRUCTURE







# EUROPEAN JOINT PROGRAMME ON RARE DISEASES & FRANCE

## Alignement of EJP RD with French initiatives

- **EJP RD will take advantage of and is aligned with:**
  - PNMR 3
  - Plan France Médecine Génomique
- **EJP RD should exploit the enormous potential of institutions, infrastructures and tools developed in France:**
  - INSERM, Imagine, IFB, universities, Orphanet, RaDiCo, national nodes of ERNs and EU Infrastructures, ANR, excellence of FR research teams...
- National mirror group will be established in order to ensure alignment and take into account FR needs and developments

# MERCI

FOR ANY QUESTIONS CONTACT: DARIA JULKOWSKA  
DARIA.JULKOWSKA@AGENCERECHERCHE.FR