European Joint Programme Cofund on Rare Diseases

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INSERM, France

ERA-LEARN thematic partnerships workshop
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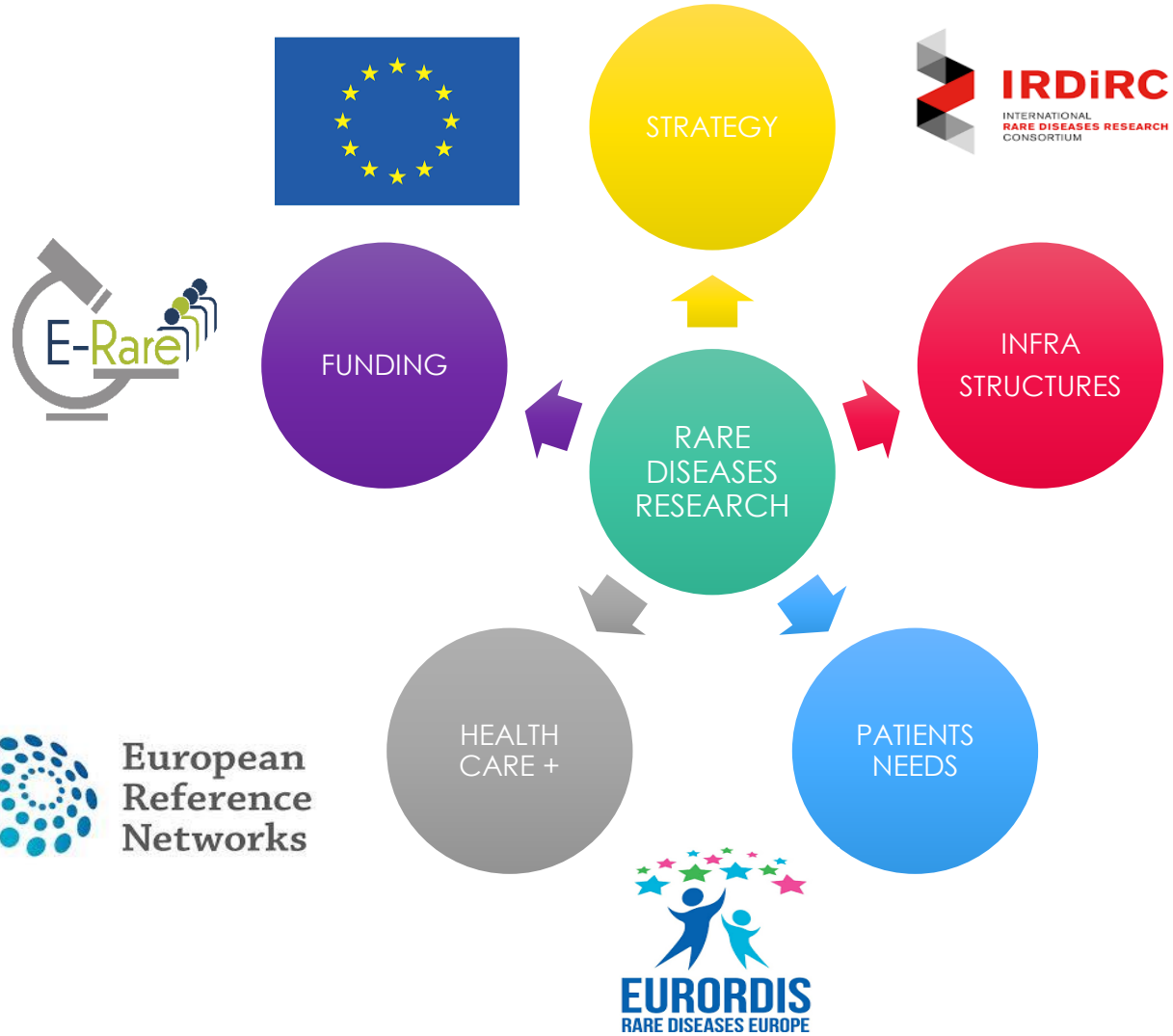
EUROPEAN JOINT PROGRAMME RARE DISEASES

Rare Diseases Landscape in Europe



INFRAFRONTIER
mouse disease models

RD Connect

ePTRI
European Paediatric Translational Research Infrastructure

orphanet

BBMRI-ERIC
gateway for health

ECRIN
EUROPEAN CLINICAL RESEARCH INFRASTRUCTURE NETWORK

Joint Research Centre
JRC

EU-OPENSCREEN
Chemical keys for life's locks

elixir

conect 4children
COLLABORATIVE NETWORK FOR EUROPEAN CLINICAL TRIALS FOR CHILDREN

eatris

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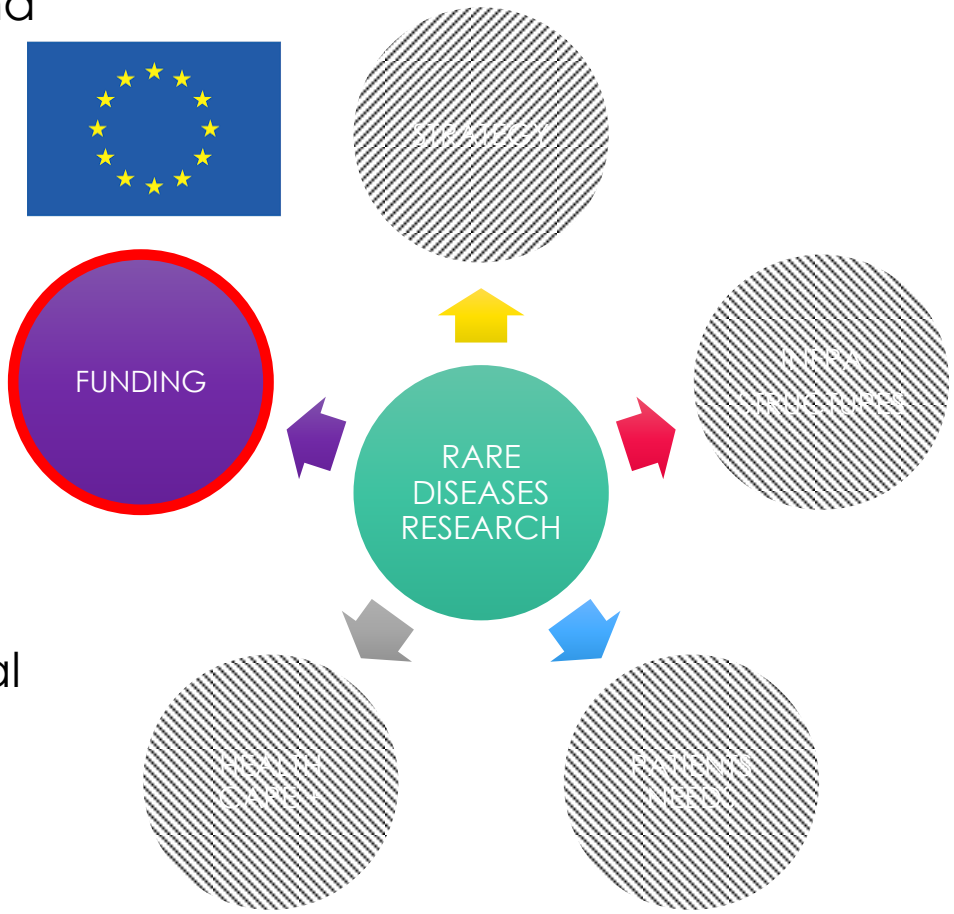
Rare Diseases Landscape in Europe

1.4 billion € in FP7 and H2020 for about 200 projects/initiatives



- 26 funding bodies from 18 countries
- Majority with NO dedicated national RD funding
- Existing since 2006
- Investment of around 100 M€ for 110 projects

INTERACTION (EC – E-Rare):

- Alignement
- Mutual exclusion/inclusion of topics of interest
- Complementarity of funding (MS & EC)
- Complementarity of size of funded consortia
- E-Rare funding considered as springboard to EC funding for research consortia

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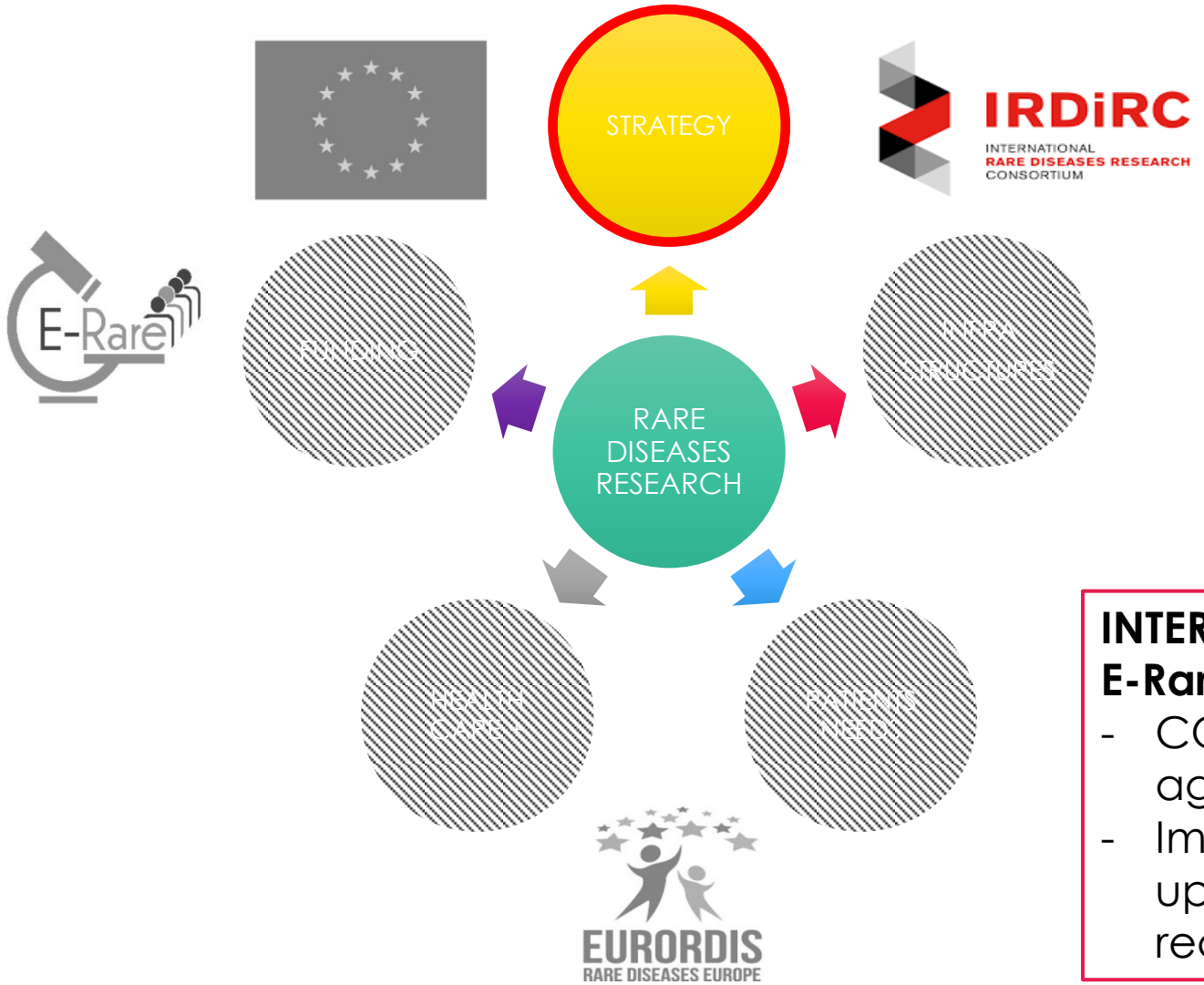

- 31 funders (including EC & E-Rare) + 11 companies + 13 patient advocates organizations (including Eurordis) representing all continents
- Strategic longer term vision & objectives

INTERACTION (IRDiRC – EC – E-Rare – Eurordis):

- COMMON strategic agenda, vision & goals
- Implementation & follow up of IRDiRC recommendations

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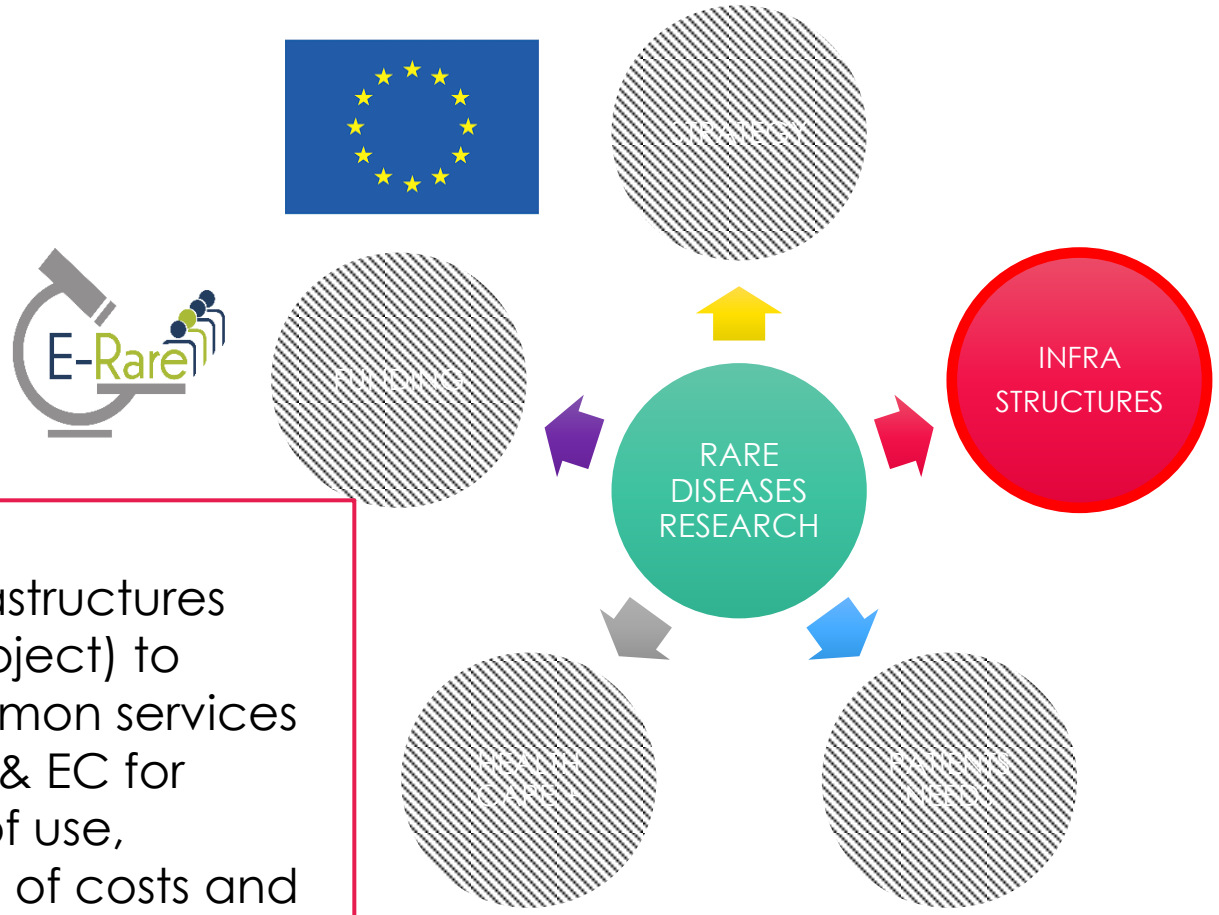

INTERACTIONS:

- Among infrastructures (CORBEL project) to create common services
- With E-Rare & EC for promotion of use, optimisation of costs and quality of research

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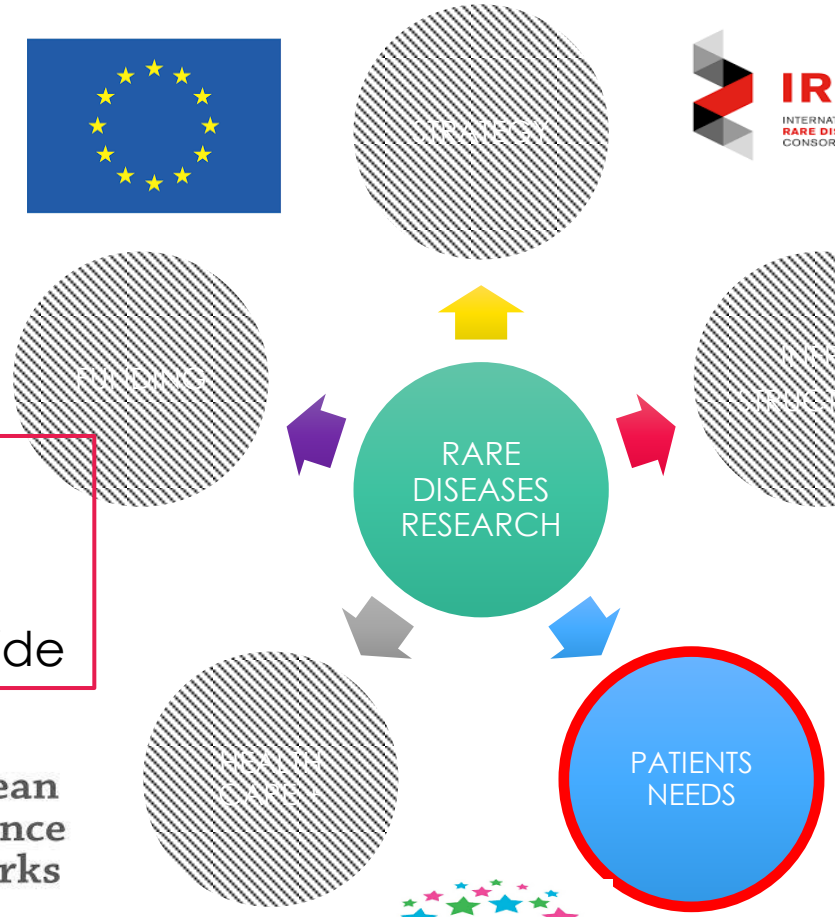
Rare Diseases Landscape in Europe



INTERACTIONS:

- Direct involvement of patients in programmes/activities/decision making

> 700 patients associations from Europe & beyond

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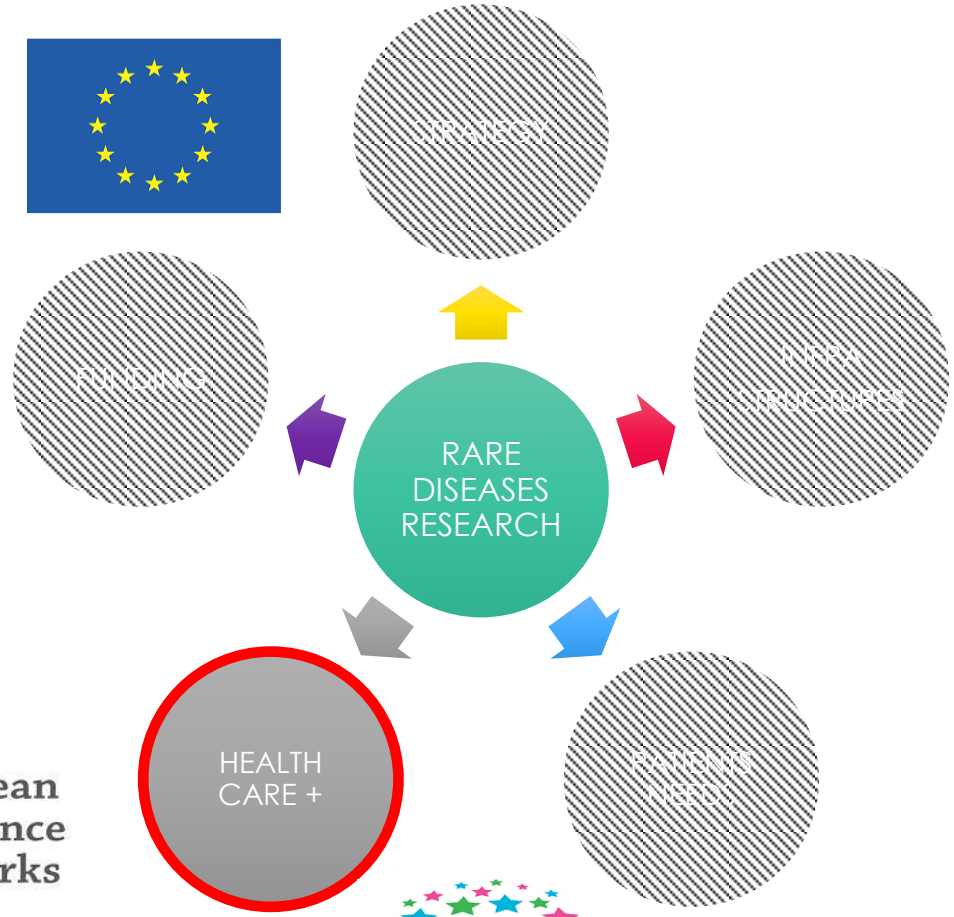
Rare Diseases Landscape in Europe



European Reference Networks

24 ERNs = 26 EU countries – 300 institutions – 900 healthcare units

EURORDIS
RARE DISEASES EUROPE

INTERACTIONS:

- Co-creation with DG Santé
- Each ERN is accompagned by its ePAG (patient advocacy group)
- Primary goal – travel of expertise and not patients
- Research among secondary objectives (initially)

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Initial thoughts/needs (E-Rare perspective)

- **Expanded funding** (budget, participating countries, type of funding opportunities) **& expanded activities** (training, mobility, capacity building)
- **Efficient translation of research results** for benefit of patients
- **Better engagement with policy makers and high-level stakeholders** (ministries, regulators, industry)
- **Stronger interaction** with and among research community and patients
- **Optimisation of use** of EU research infrastructures
- **Sustainability**

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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

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Objectives

- *Enable all people living with a rare disease to receive an accurate diagnosis, care, and available therapy within one year of coming to medical attention (IRDiRC overarching vision)*
- **Integrate existing**
- **Connect existing – common RD research ecosystem**
- **Exploit and enhance potential of individual elements**
- **Build new tools, methods, funding schemes for more efficient research**
- **Optimise resources & create strong interaction between national-EU-international strategies**
- **Open science**

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Why EJP Cofund?

Features	ERA-Net Cofund	EJP Cofund	Art.185 Initiative
Level of integration	Low	High	High
Who can participate	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
Who signs	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 Commisison and DIS (Deignated implementation Strcuture)
Open joint calls	Yes	Yes	Yes
Central grant management	Not necessary but possible	Not necessary but possible	Yes (no involvement of funding agencies expected)
Direct research activities	No with exception of in kind ERA-Net	Yes	Yes
Annual work plan	No	Yes	Yes
Type of actions possible to include	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
Reimbursement rate	Max. 33%	Negotiable (50 - 70%)	Max. 50% COM contribution to costs of the programme

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EJP RD preparatory phase

- 2 years of discussions and adjustments
- Progressive inclusion of stakeholders (working group of experts → invitation of all MS →invitation of other stakeholders)
- 2 concept drafts, 3 F2F meetings (>100 participants), 4 months of intense writing by core group (35 people) + 3 draft consultations/revisions by all partners (250 people)
- In depth analysis of existing projects/programmes/infrastructures/trainings and strategic choices on what brings added value
- Invitation of pre-identified resources and selection of other contributors via LOIs
- Challenging integration of networks/multi-partner with no legal status (ERNs, Orphanet)
- Drastic discussion on budget (elimination of activities, more [in kind] effort demanded)
- Constant strong support from the FR ministry (help in negotiation with other MS)
- Core operating group (10 persons), strongly dedicated and with v. good knowledge of RD landscape and EC schemes

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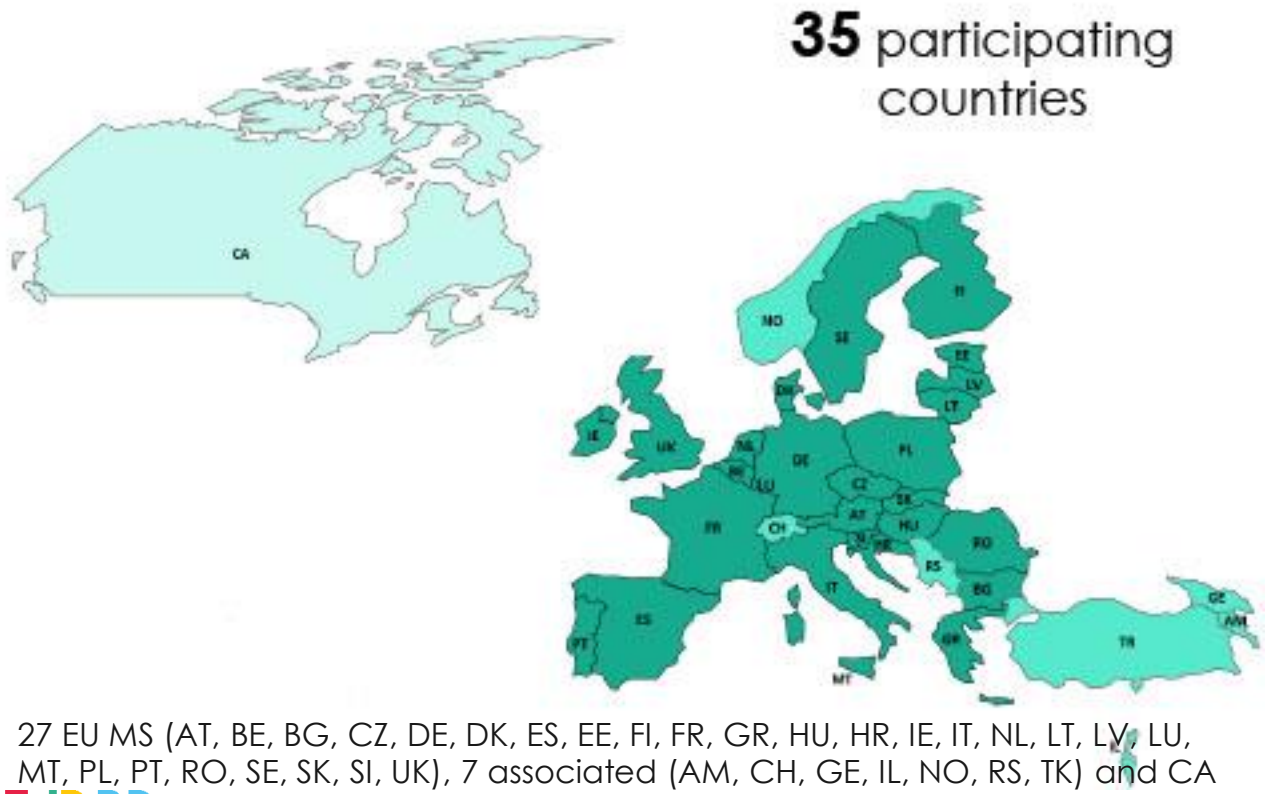
Main facts about the EJP RD

Jan 2019 → **Dec 2023**

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

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



27 EU MS (AT, BE, BG, CZ, DE, DK, ES, EE, FI, FR, GR, HU, HR, IE, IT, NL, LT, LV, LU, MT, PL, PT, RO, SE, SK, SI, UK), 7 associated (AM, CH, GE, IL, NO, RS, TK) and CA

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

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EJP RD STRUCTURE

Coordinated by Inserm
La science pour la santé
From science to health

COORDINATION
& TRANSVERSAL ACTIVITIES

INTEGRATIVE RESEARCH STRATEGY

SUSTAINABILITY

ETHICAL & REGULATORY

COMMUNICATION

1 FUNDING

2 COORDINATED ACCESS TO DATA & SERVICES

3 CAPACITY BUIDLING & EMPOWERMENT

4 ACCELERATING TRANSLATION OF RESEARCH & THERAPY DEVELOPMENT

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Share of partners per Pillar

112 LOI received representing:

- 25 countries (AT, AU, BE, CA, CH, CZ, DE, DK, EE, ES, FR, HU, GR, IE, IL, IT, LT, LV, NL, PL, PT, SE, TK, UK, USA)
- 24 ERNs
- 31 « international » organisations (infrastructures, EURORDIS) + 3 charities

Share of interest between pillars:

- P0: 19 institutions representing all stakeholders (funders, research performing organisations, infrastructures, hospitals, patients and charities
- P1: 31 institutions from 22 countries (AT, BE, CA, CH, CZ, DE, EE, ES, FI, FR, HU, GR, IE, IL, IT, LT, LU, PL, PT, SE, SK, TK) + EURORDIS + charities
- P2: 71 institutions from 18 countries (AT, BE, CH, DE, DK, EE, ES, FR, HU, IT, LV, NL, PL, PT, SE, SI, TK, UK) + ERNs + international (infrastructures)
- P3: 51 institutions from 16 countries (AT, CH, CZ, DE, EE, ES, FR, HU, IE, IT, LT, LV, NL, PL, PT, TK) + international (EURORDIS; infrastructures) + ERNs
- P4: 32 institutions from 9 countries (BE, CH, ES, FR, IT, NL, TK) + ERNs + charities + international (EURORDIS; infrastructures)
- AU & USA collaboration ensured first via IRDiRC

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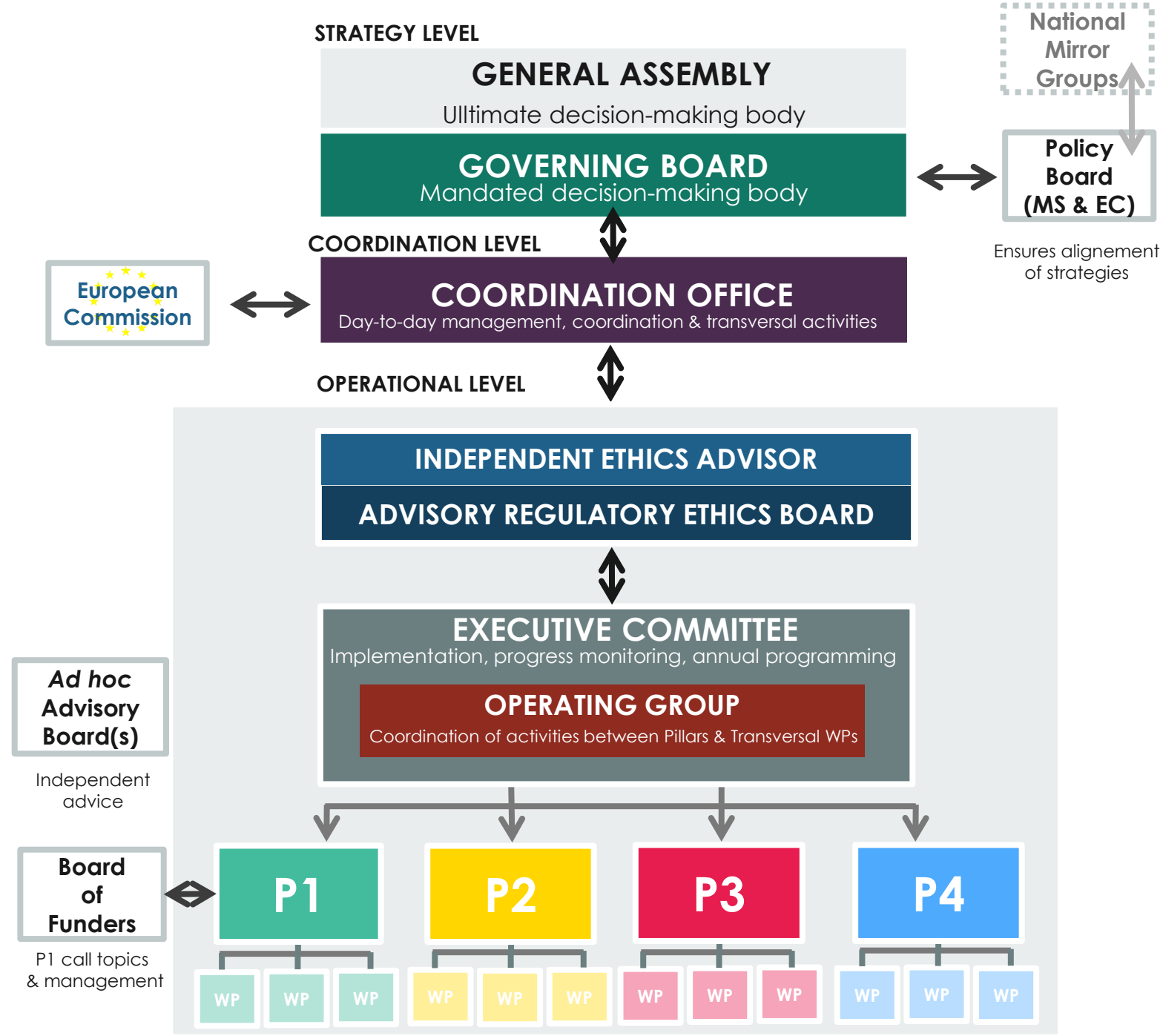

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POLICY BOARD & BOARD of FUNDERS

- The **POLICY BOARD** will have a major role in ensuring this dialogue and translation through its participation is EJP RD strategy and sustainability development. It will meet once a year.
- The Policy Board will be constituted from:

  - Representatives of national ministries of research and health;
  - Representatives of European Commission Directorates: DG RTD, DG Santé, DG Connect;
  - Representative of the pharmaceutical industry and public-private initiatives (e.g. EFPIA, IMI, EUCOPE, EuropaBio);
  - Representative of regulatory authorities (e.g. European Medicines Agency, EMA, esp. Committee for Orphan Medicinal Products, COMP, EuNetHTA);
  - Chair of the European Strategy Forum on Research Infrastructures (ESFRI);
  - Chair and vice-chair of the International Rare Diseases Research Consortium (IRDiRC).

- **BOARD OF FUNDERS:**
- In order to ensure the independence of joint transnational calls management (Pillar 1), the final decisions on call topics and implementation of calls will be taken autonomously by the Board of Funders (BoF). The composition of Board of Funders will be variable depending on the configuration of funding bodies participating in the joint transnational calls. BoF will be chaired by the Pillar 1 leaders.

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NATIONAL MIRROR GROUPS

It is up to each participating country to decide on how to establish a common voting position on each agenda item, so that the vote faithfully represents a consensual national position and not the one of any specific institution.

- **NATIONAL MIRROR GROUP:**
- Participating countries will be strongly advised to constitute NMG, bringing together the national representatives of the EJP RD and other relevant RD stakeholders. The creation and composition of a NMG is at the discretion of each participating country. Although not mandatory, it is expected that the establishment of National Mirror Groups will ensure that national activities, strategies and needs are taken into account when taking decisions at the EJP RD level and when designing the annual work plans.

- NMG ensures national coordination, contribute to the objectives of the EJP RD and benefit from it
- Is expected to include representatives of the National plan for RD, national nodes of the European Reference Networks, relevant national authorities and research institutions (whether participating to the EJP RD or not), as well as the relevant national partners of the EJP RD and **GB member** that will report NMG views and positions during GB meetings.

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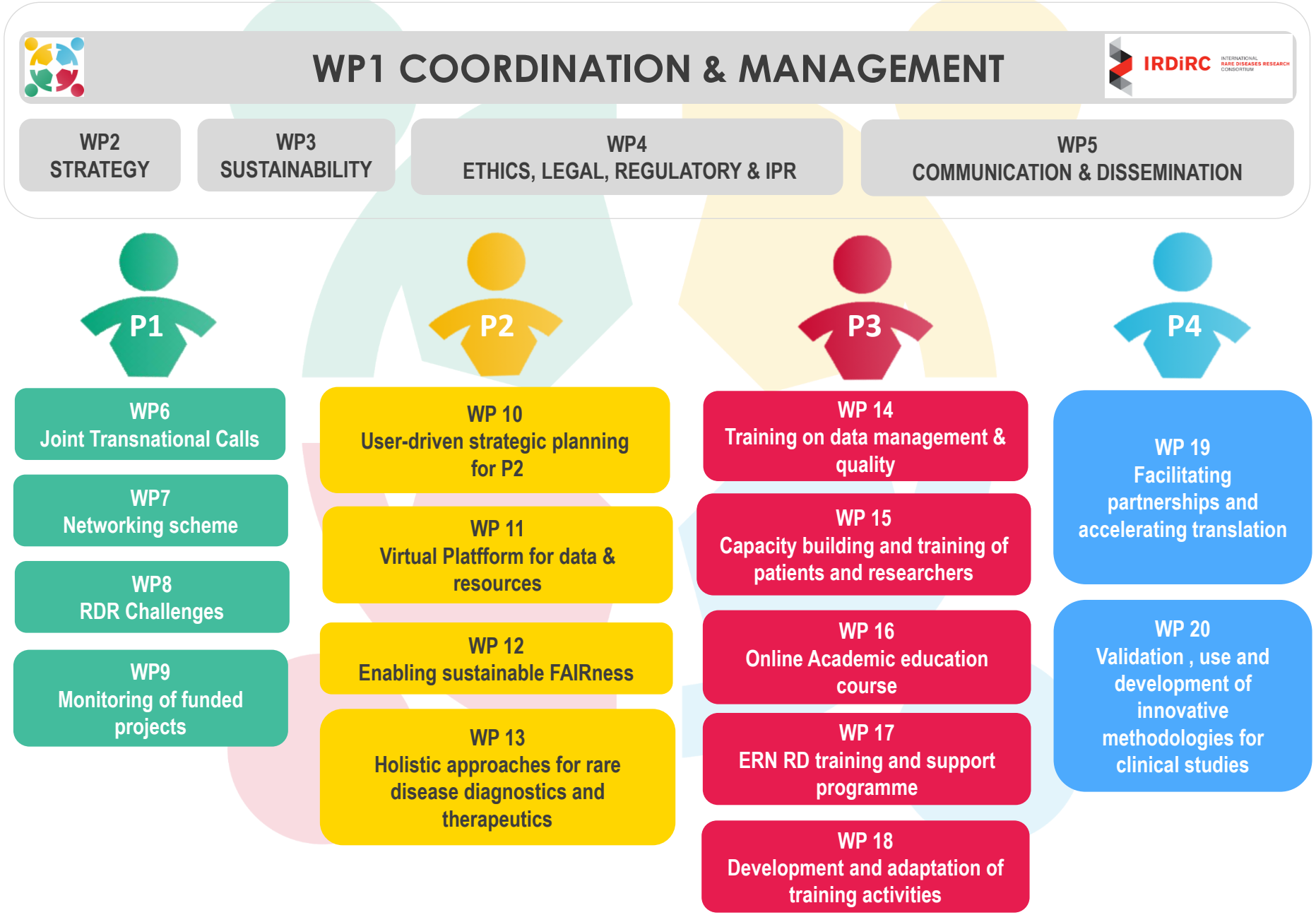
Why together is better than separately?



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Why together is better than separately?

- **Expanded funding support** → for collaborative research projects (ERA-Net type), for enhances networking & share of knowledge (COST type), for innovation (with industry), for patients (direct support of patient associations)
- **Virtual platform of resources, data & services** → centralised service for collections (biobanks, registries, infrastructures and tools catalogue, analysis platform for omics data), federated service for data (FAIR, single access, protections, discoverability)
- **Enhanced training capacity** → boosting of existing trainings of value, creation of new programmes (accredited academic programme for RDs open to all, for researchers, patients & healthcare personnel, mobility programmes, knowledge sharing & travelling to EU 13)
- **Faster innovation** → innovation management of funded projects, direct access to derisking & sponsors, new models for RD funding
- **Dialogue with regulators** → innovative methodologies for clinical trials in small populations (new endpoints, new statistical approaches)
- **Increased EU competitiveness** → high quality research, immediate access to expertise & resources, dedicated services (mutlinational CTs; innovation managers)
- **For & with society** → patients involved at all levels, healthcare economy & social impact measures
- **Alignement of national, EU & international strategies** → dialogue with NMG, key involvement of Policy Board, building of sustainability from the start

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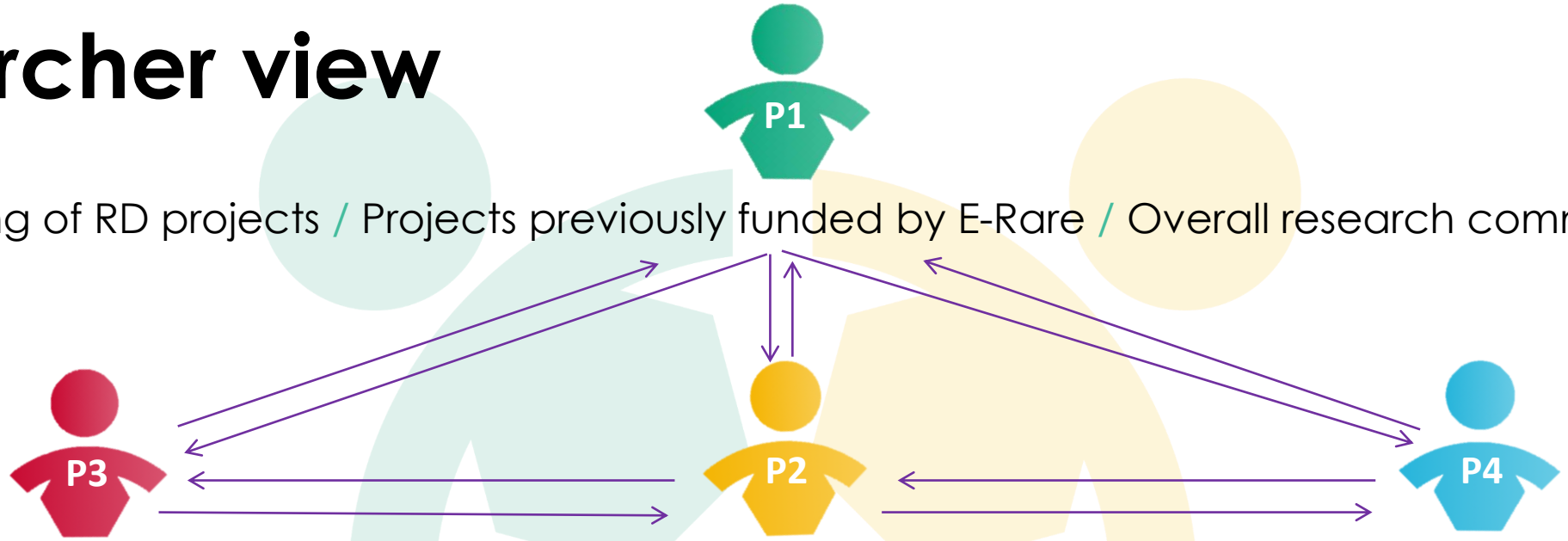
Researcher view

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

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What about increased coherence & complementarity with other existing partnerships?

- **EJP RD & other P2Ps:**
  - Strong interaction with EU infrastructures
  - Loose collaboration with other programmes → mainly through inclusion/exclusion of topics in calls for projects and sharing of best practices (guidelines or practices developed by other programmes)
  - No merge of pre-existing P2Ps but involvement of all relevant stakeholders
- **EJP RD in Horizon Europe – challenges & opportunities:**
  - New or remaining challenges:
    - Data sharing → still requires major efforts to decrease overlaps
    - (rare) cancer research → challenge for current models/organisation
  - Use of EJP RD results as proof of concept for enhanced collaboration/alignment with private partnerships
  - Use of EJP RD results as proof of concept for enhanced knowledge and innovation → collaboration with KICs

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Main challenges vs main advantages of EJP instrument

- **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
  - Needs further improvement for planning & reporting → Annual Work Plans are nightmare as timeline not adapted (1st AWP to be delivered on M9!; reporting tools do not integrate the dynamic character of the programme leading to continues amendments)
  - Possible "sophisticated" financial arrangement → depending on the choice of the financial approach (e.g. black box model)
  - The problem of beneficiary vs third party status for open calls for projects → conflicting status of RPOs

- **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%
  - 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

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THANK YOU

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