



Rare Diseases Partnership

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Online

European Partnerships – Why?

Horizon Europe will support the next-generation of European Partnerships to **deliver on global challenges** through concerted R&I effort with the Member States, private sector, foundations and other stakeholders.

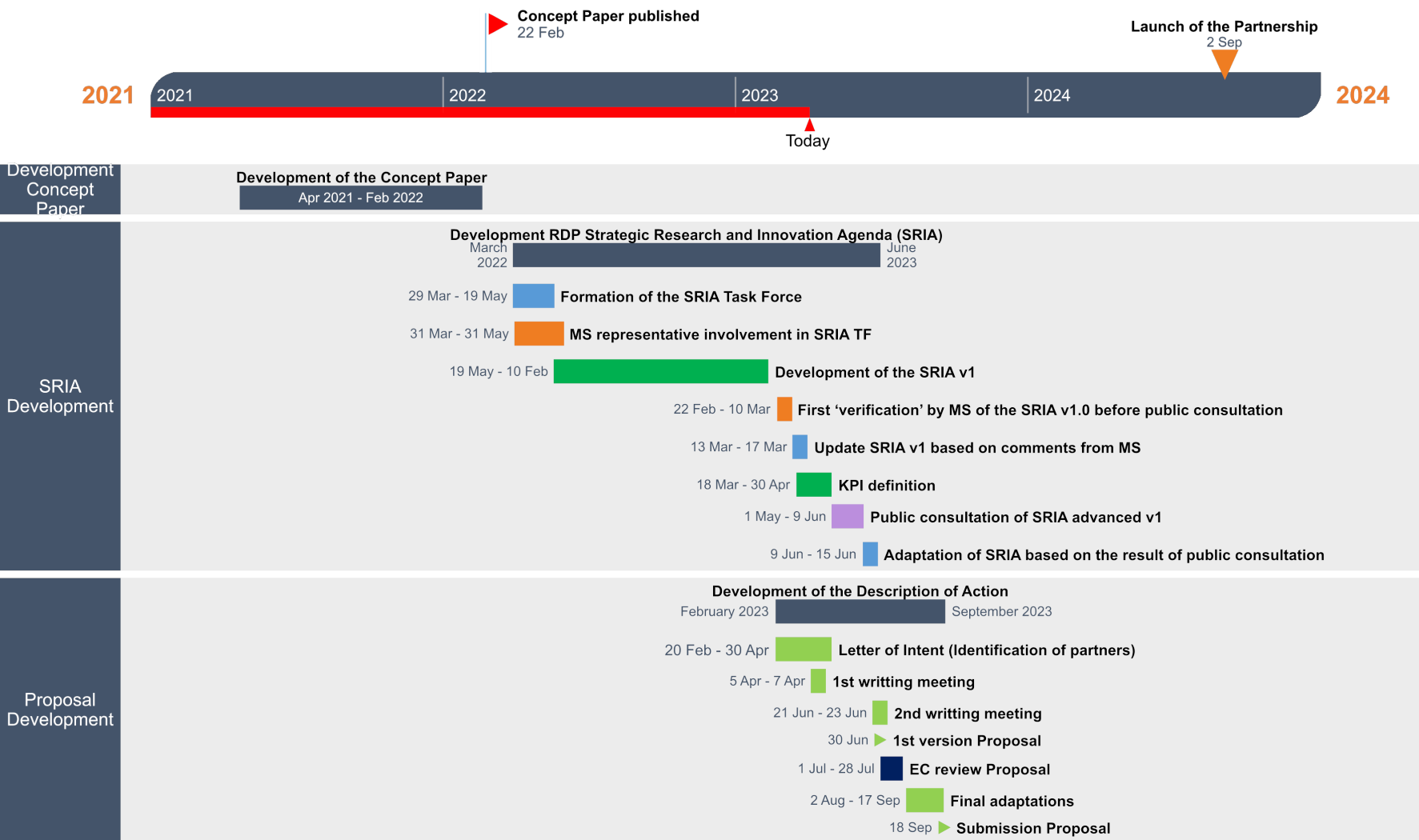
Horizon Europe expects partnerships to take a “**systemic approach in the achievement of the objectives**”, including “to ensure coordination with other relevant R&I initiatives”.

European Partnerships

- provide mechanisms to link R&I closely to policy needs
- develop close synergies with national and regional programmes
- bring together a broad range of innovation actors to work towards a common goal
- turn research into socio-economic results



Rare Diseases Partnership



This timeline is subject to change (last update 22-02-2023)



STRATEGY

International Rare Diseases Research Consortium (IRDiRC), EC, Member States

INFRASTRUCTURES

Orphanet, RD Connect, ERDRI, Solve-RD, EATRIS, ECRIN, BBMRI, etc.

FUNDING

ERA-Net Rare (2006-2018) + EC + industry

PATIENTS NEEDS

EURORDIS

HEALTHCARE +

European Reference Networks (ERN)

RESEARCH ECOSYSTEM

EJP RD



**WHAT ARE THE
REMAINING
NEEDS?**



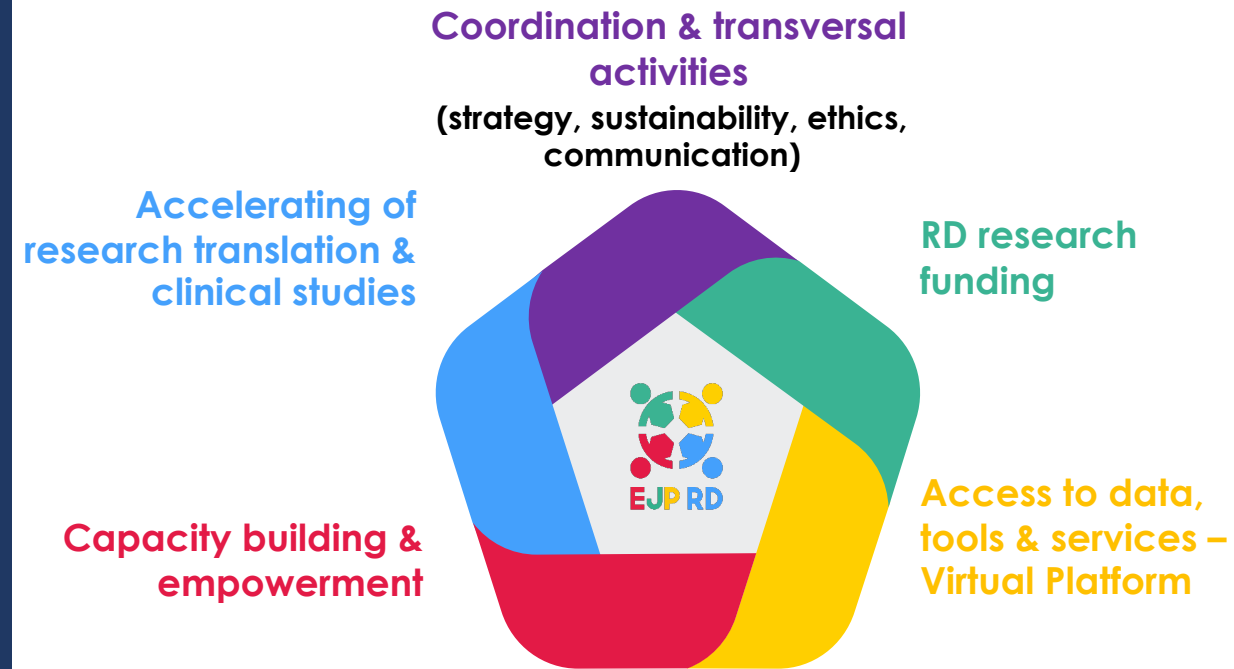
European Joint Programme on Rare Diseases – objectives & structure

🧩 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

🧩 Mode of action:

Large programme that integrates existing infrastructures, trainings, funding programmes and tools, expands them and develops new essential ones to offer harmonized (and centralized) RD research ecosystem that is easy to use for scientists and produces benefits for patients in the most efficient way



+2300
people

35 participating
countries

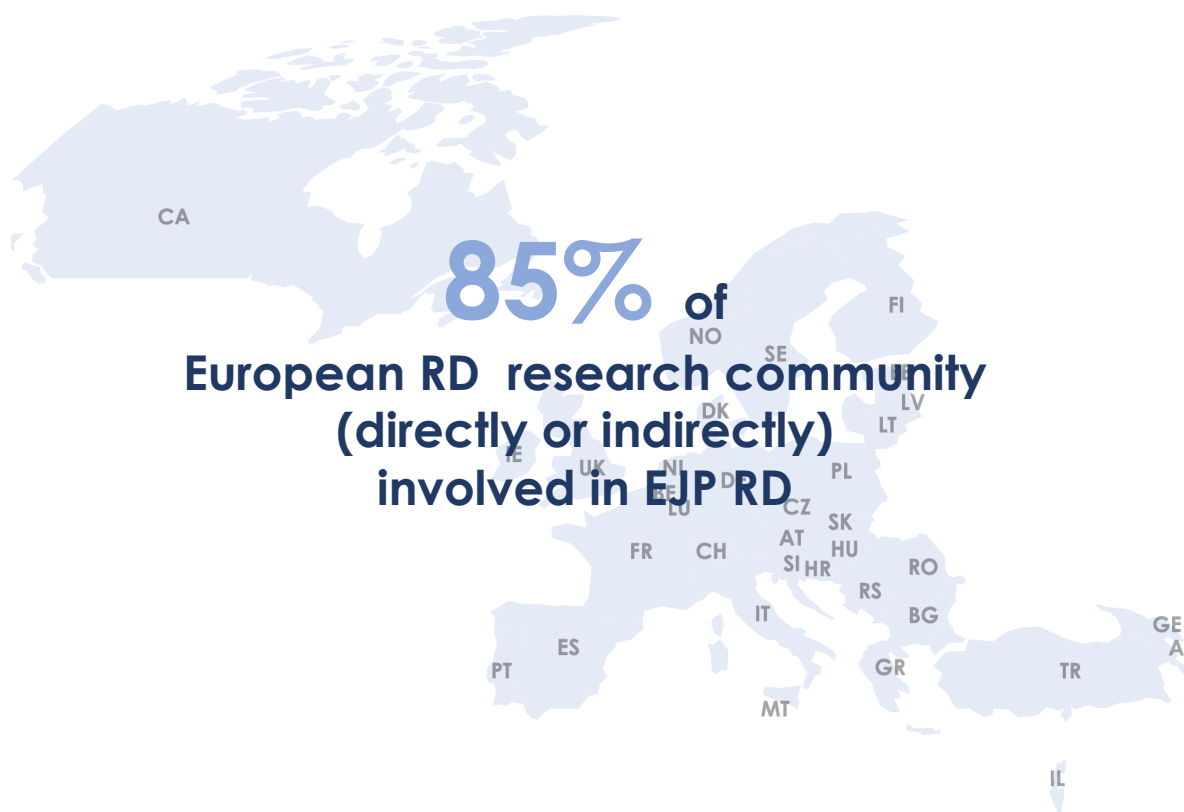
26 EU MS, 7 associated (AM, CH, GE, IL, NO, RS, TK), UK and CA

ALL 24 ERNs

101 M€
Budget

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

EJP RD in numbers



94 beneficiaries

10 hospitals

13 research institutes

31 research funding
bodies/ministries

29 universities/hospital universities

5 EU infrastructures

5 charities/foundations
EURORDIS

+ 47 linked third parties

+100% associated
networks

EJP RD – single entry point & solutions for all

RESEARCHERS



Funding

Research support services

Training at every stage

Access to resources & tools

Access to extensive network & expertise

CLINICIANS



Clinical studies support services

Support for registries

Access to resources & tools to accelerate diagnosis

Access to extensive network & expertise

Funding

PATIENTS



Access to RD specific expertise

Networking

Training at every stage

Access to resources & tools

Access to extensive network & expertise

Funding

POLICY
MAKERS &
FUNDERS



Joint funding & strategy

Optimisation of investment in research

Access to support for national RD community

Access to extensive network & expertise

Holistic impact evaluation

INTERNATIONAL
PARTNERS



Access to extensive RD network & expertise

Multiple collaboration opportunities

Possibility of alignment

Access to resources & tools



HOW EJP RD EMPOWERS STAKEHOLDERS & PERFORMANCE OF RESEARCH ACTIVITIES?



Networking support to build ideas, engage patients, align needs

Profit from multinational funding opportunities



Follow on funding support

Clinical studies support office

Knowledge sharing



Use VP to find suitable tools (models, screening, biosamples, pathways for drug targets)

Benefit from expert mentoring support at application stage



Mentoring follow up

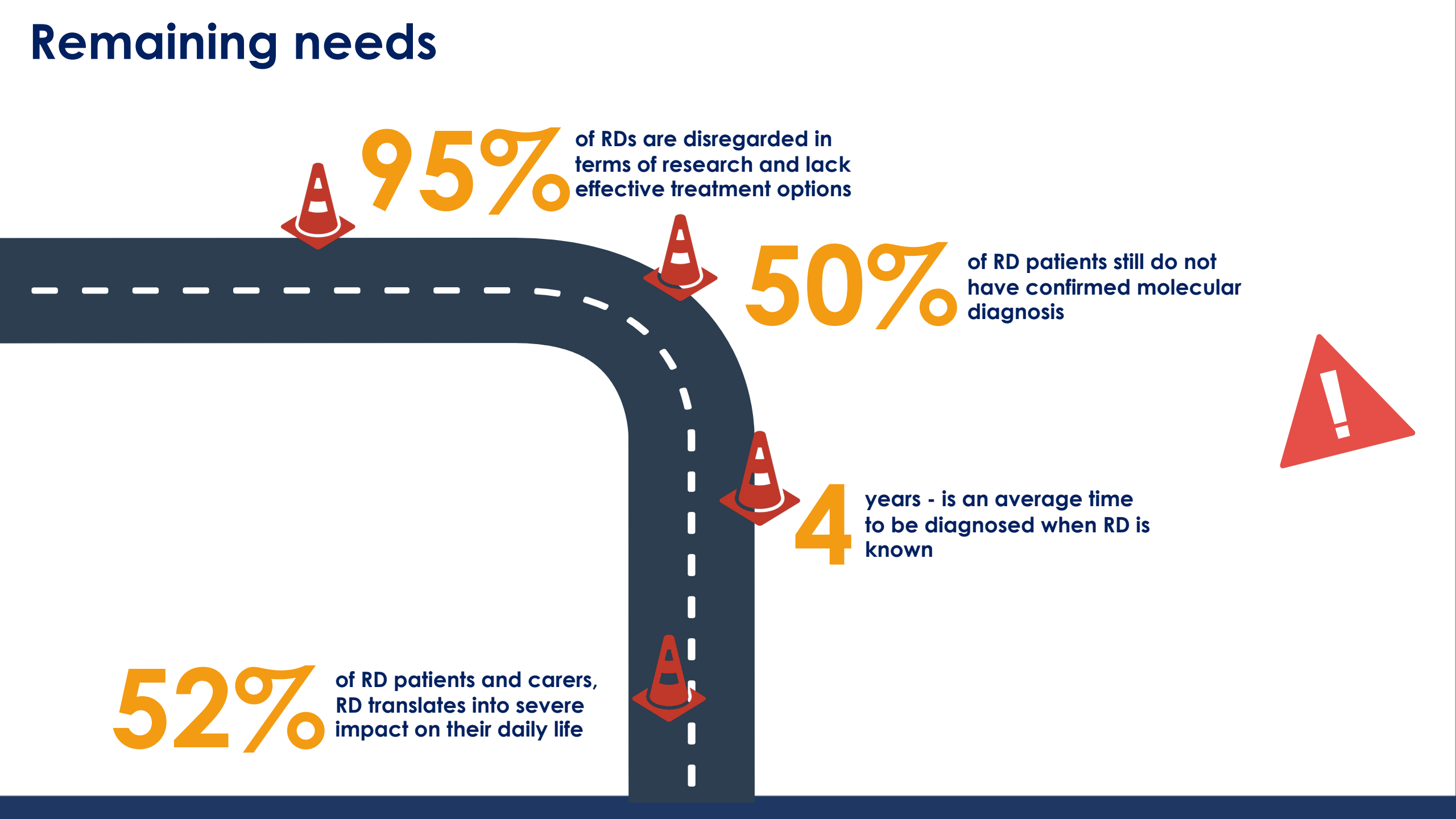
Training for PIs & students

Data FAIRification

Tools for phen-gen analysis



Remaining needs



95% of RDs are disregarded in terms of research and lack effective treatment options

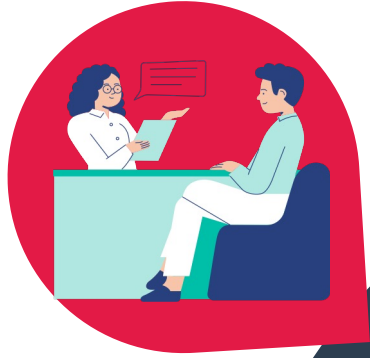
50% of RD patients still do not have confirmed molecular diagnosis

4 years - is an average time to be diagnosed when RD is known

52% of RD patients and carers, RD translates into severe impact on their daily life

SUPPORT

robust patient need-led research



UTILIZE

the power of health and research data and spearhead the digital transformational change in RD research and innovation

Rare Diseases Partnership Vision



DEVELOP


new treatments and diagnostic pathways



SUPPORT

the coordination and alignment of national and regional research strategies, including the establishment of strong public-private collaborations

Rare Diseases Partnership Mission



Bring supporting R&I services from across Europe under one roof so that every high-quality RD research project will benefit from cross-disciplinary expertise, goal-oriented study planning and efficient execution

Enable every consenting patient living with a rare disease to be findable and enrolled in a suitable clinical study, by boosting generation of regulatory-level and FAIR-compliant data from diversity of sources, with the ultimate goal to fasten advances in prevention, diagnosis, disease knowledge and treatment

Make Europe a global leader on rare disease research through a significant increase in investment to spur innovation, leading to job creation and improving EU competitiveness in R&I

Competitive Research Funding and Transversal Support Services

Meaningful and exploitable results

Networking Support Scheme

Patient Engagement Facilitation



Capacity building



Annual Joint Transnational Calls

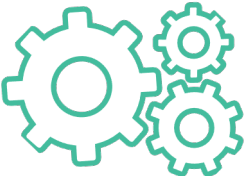


Mentoring service



Acceleration hub

Data integration and coordination services



Ethics & regulatory Support

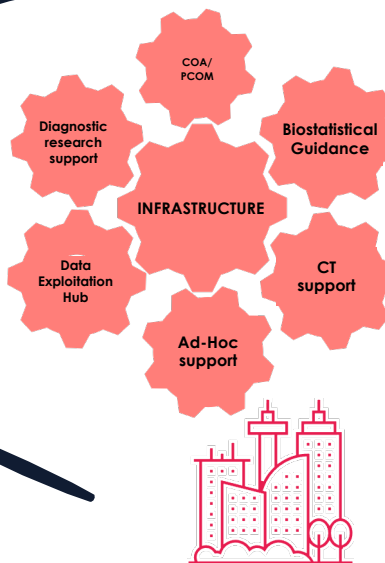


Clinical Research Network

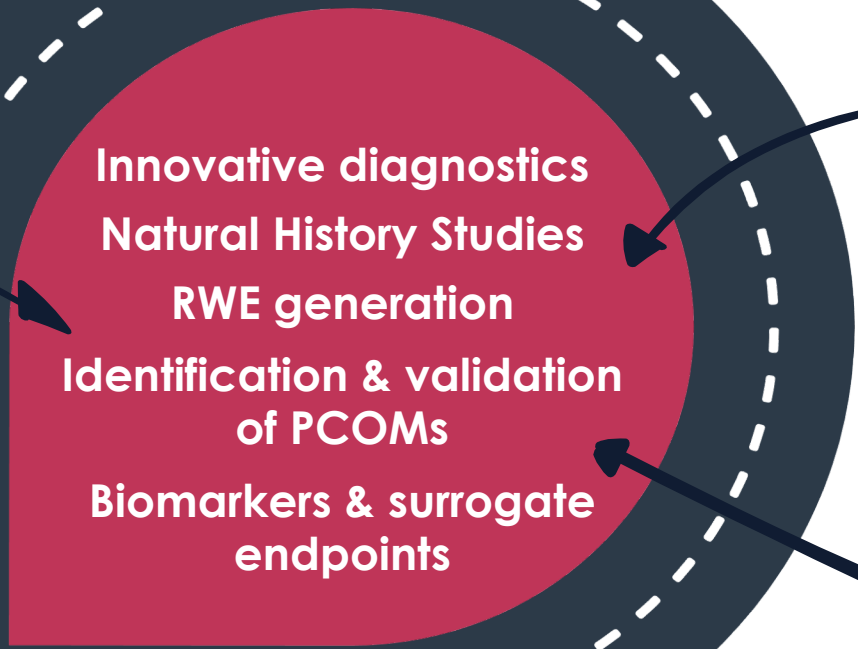
National alignment & capacities



Technological expertise



Infrastructure



Public-private collaboration

Accelerated diagnosis
Clinical trial readiness
New therapies

EUROPEAN CLINICAL RESEARCH NETWORK FOR RARE DISEASES

RD Clinical Research Infrastructure
Data exploitation hub
Diagnostic research support
COA/PCOMs support
Biostatistical guidance
Clinical trials support



COMPETITIVE RESEARCH FUNDING AND SUPPORT

Joint Transnational Calls,
Networking

TRANSVERSAL SUPPORT SERVICE

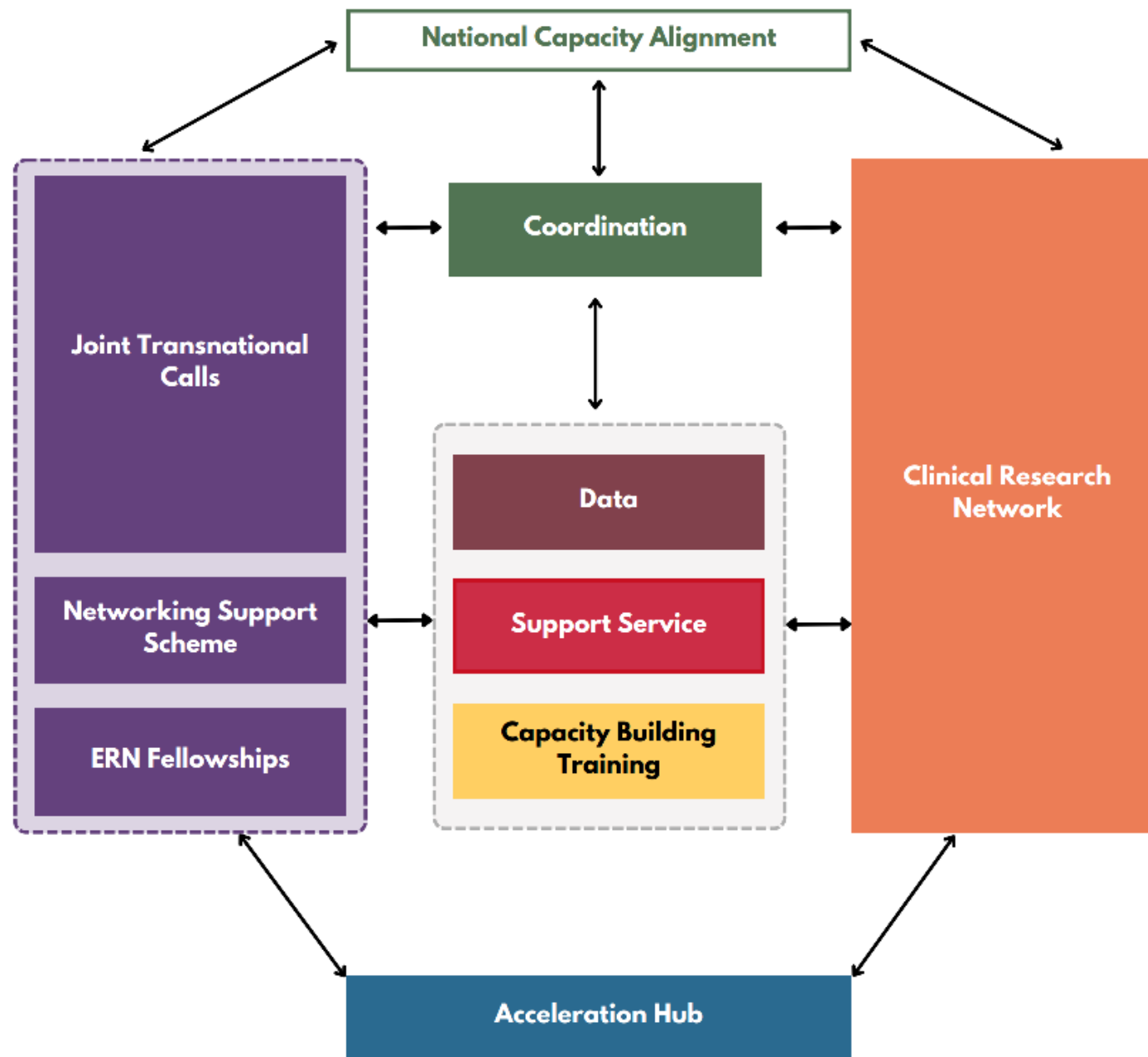
Mentoring service
Data integration
Capacity building
Ethics & regulatory support
Acceleration hub



COORDINATION, STRATEGY, GOVERNANCE

Public-private collaboration
Maximisation of national
alignment & contributions
Joint multi-stakeholder strategy
Patients as drivers







Opportunities for SMEs

Opportunities for SMEs

- Applicants/participants in research projects financed within Joint Transnational Call, Networking Scheme opportunities and Acceleration Hub
- As direct beneficiaries in the RDP e.g.:
 - Providing specific tools/technology/performing research in CRN
 - Involved in training/education activities
 - Involved in mentoring support service as advisors
 - Contributing to the governance
- As users of RDP services (mentoring, acceleration hub, ELSI, training, etc.)

Questions to you

- What would be your expectations toward RDP? How it can help you? (*to engage for the benefit of RD patients*)
- What are the ways of possible involvement?
- What are the obstacles to your involvement?
- Are there any specific (funding/support) schemes that you know and that could be connected to increase the funding opportunities for SMEs in RDP? (e.g. Innovative SMEs Partnership)
- In case RDP offers interesting ways of involvement/funding what would be the topics you would be interested in (e.g. diagnostics, AI, etc.)

THANK YOU

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