CONNECTING THE DOTS IN THE RARE DISEASES RESEARCH ECOSYSTEM

THE EUROPEAN RARE DISEASES RESEARCH ALLIANCE (ERDERA)

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ERN-SKIN webinar
16/01/2024 _ Online

ERDERA

EUROPEAN **RARE DISEASES** RESEARCH ALLIANCE

2024 – 2034

ERDERA proposal **was submitted under Horizon Europe** on 19 Sept. 2023 for funding under the EU Research & Innovation funding programme Horizon Europe as a co-funded partnership between the European Commission, European Member States, and beyond.

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Planned activities

Open until 22/01/2024

Go to: **menti.com**

Enter the Code: **4733 2222**

Or click on this link: https://www.menti.com/ala5yuthf51z

How could they be best performed?

What should they consider?

How could your current and upcoming activities be linked (to them)?

Please be precise

Provide details & links

You feedback will be processed.

The summary will be presented during the discussion session.

The details will be exploited for preparing ERDERA launch & activities implementation.

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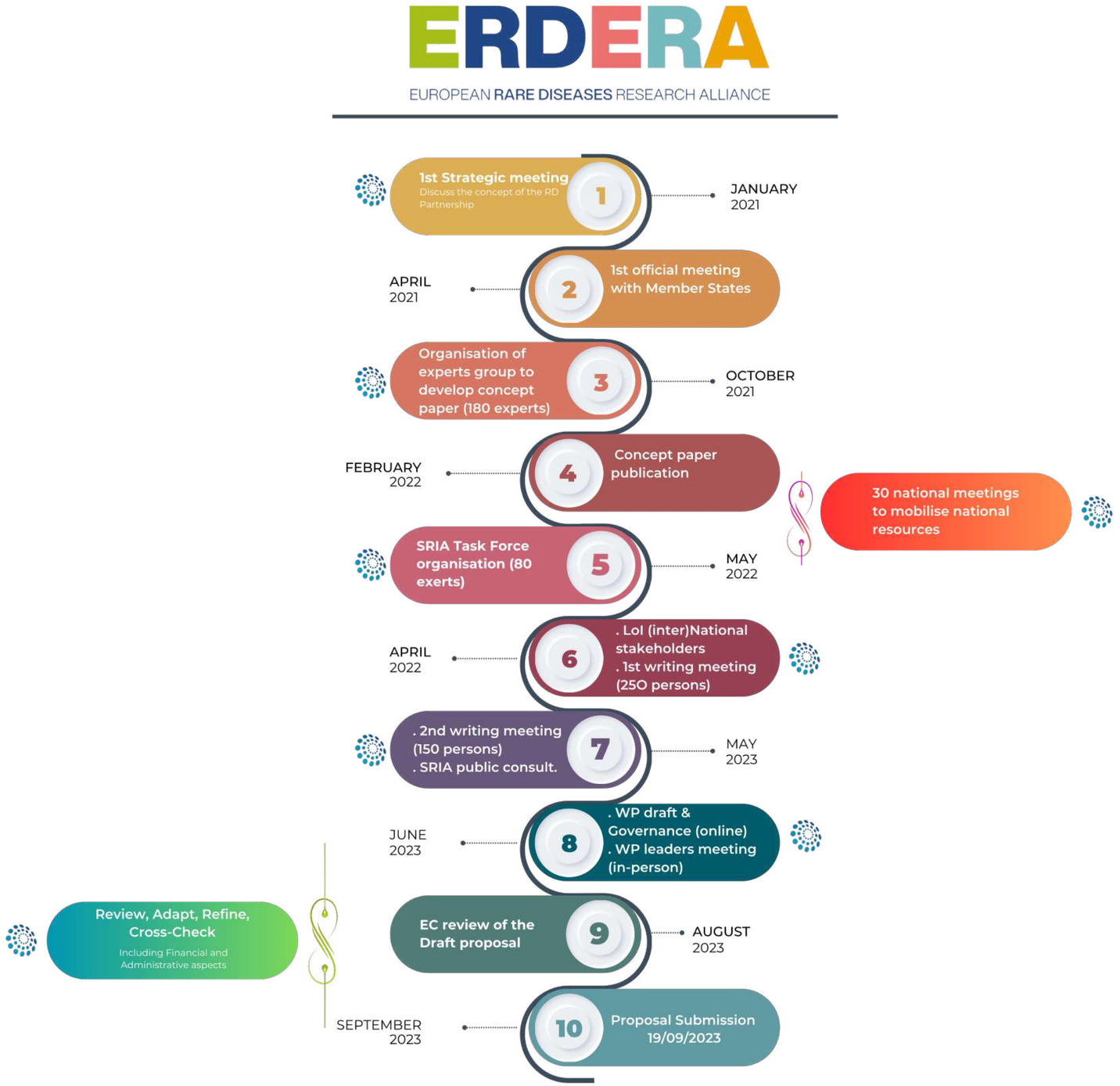
Timeline

https://tinyurl.com/5yev82yj



RD Partnership timeline _ in short

- End 2019: Validation of RD Partnership as part of the HE
- Jan 2021: first strategic meeting to discuss the concept
- Apr 2021: first official meeting with Member States
- Oct 2021: organisation of experts group to develop concept paper (180 experts)
- Feb 2022: publication of ERDERA concept paper (validated by the EC)
- Feb-Apr 2022: 30 national meetings to mobilise national resources
- May 2022: organisation of SRIA Task Force (80 experts) → May 2023 SRIA opened for public consultation
- Apr 2023: request for Letters of Intents from national & international stakeholders
- 5-6 of April 2023 – 1st writing phase meeting
  - 250 people, open to all, organisation in session, sharing of ideas to start forming the WPs
- 2nd of May 2023 – 2nd writing meeting
  - 150 people, open to representatives from interested organisations, 6 parallel sessions covering pre-defined groups of activities, proposition of WPs
- May 2023: organising working groups for each WP and start of the writing process
- June 1st: online meeting to present the first draft of the WPs + session dedicated to the discussion on governance
- June, writing continues
- 21-23 of June, in person writing meeting to finalise the complete draft of the proposal
- Summertime: finalisation of the admin aspects, feedback from the EC and the identified ERDERA contributors, focused WorkStreams and WP meetings meetings
- 18 of Sep: submission

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171
Organisations*

38 funders

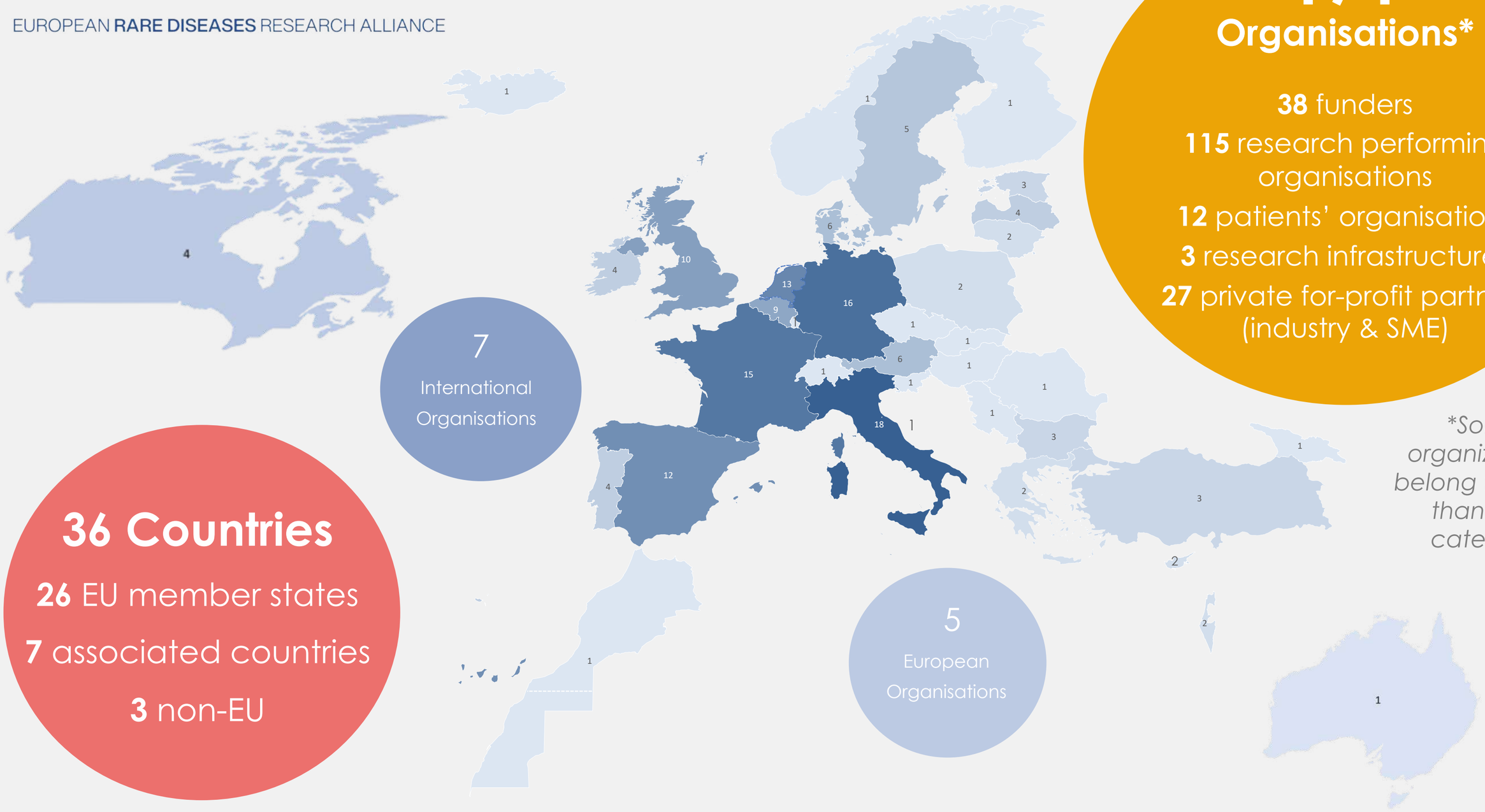
115 research performing organisations

12 patients' organisations

3 research infrastructures

27 private for-profit partners (industry & SME)

**Some organizations belong to more than one category*



7
International Organisations

5
European Organisations

36 Countries

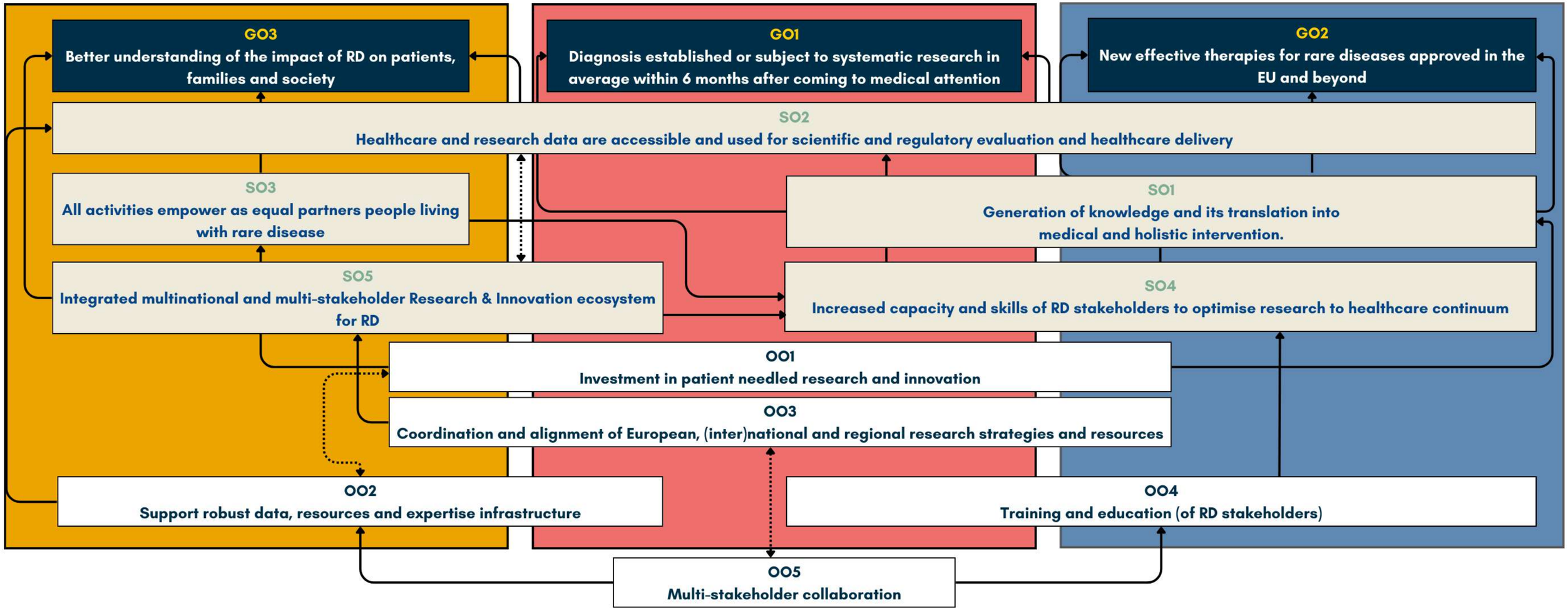
26 EU member states

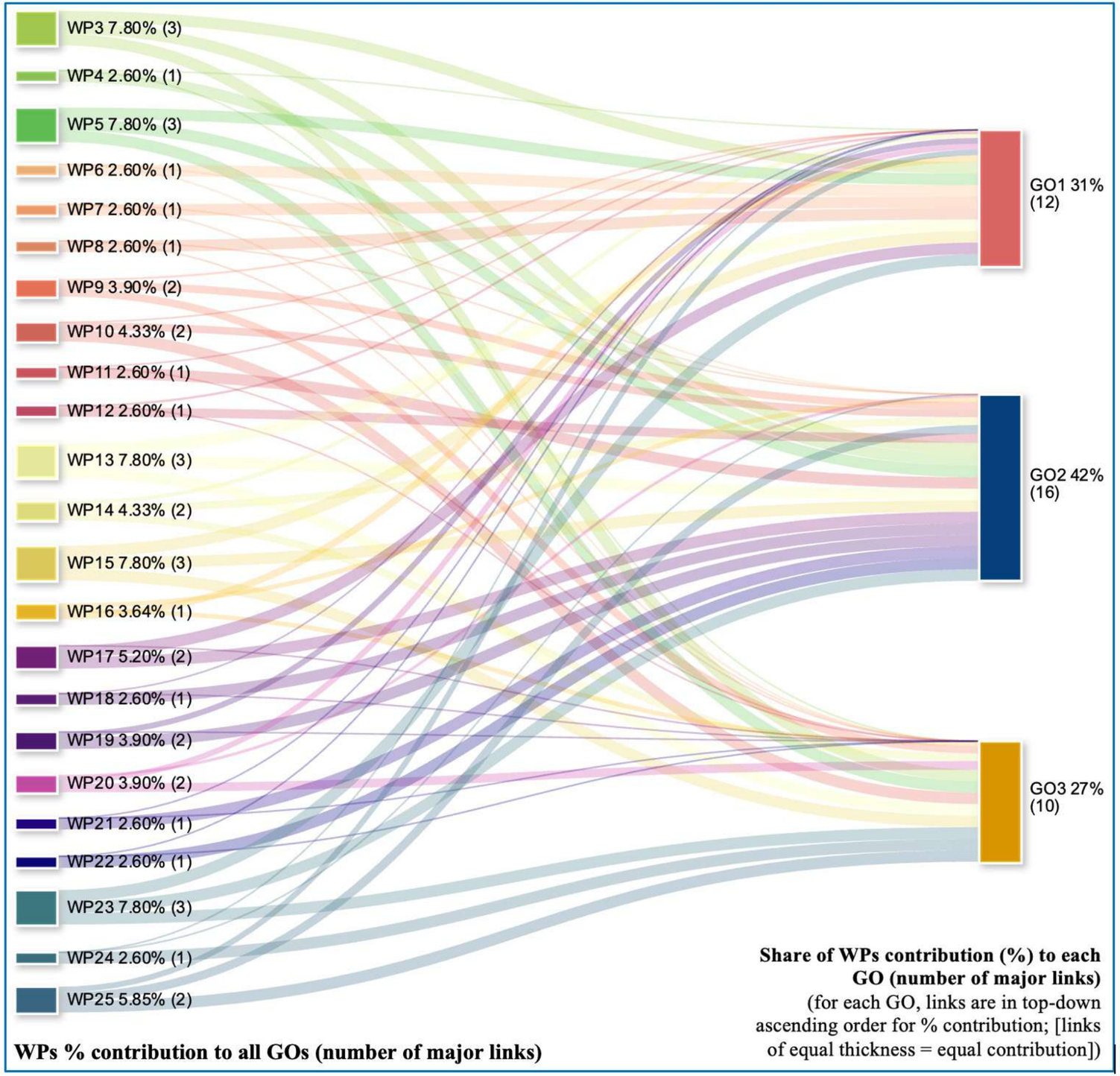
7 associated countries

3 non-EU

PARTNERSHIP SPECIFIC IMPACT PATHWAYS

VISION: To improve the health and well-being of 30 million persons living with a rare disease in Europe, by making Europe a world leader in RD research and innovation, and delivering concrete health benefits to rare disease patients, through better prevention, diagnosis and treatment.





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Diagnosis established or enrolment in systematic research on average within 6 months after coming to medical attention (in EU and Associated countries)

New effective therapies for rare diseases approved in the EU and beyond

Better understanding of RD impact on patients, families and society

Work Package relative contribution to the General Objectives based on the number of tasks involved

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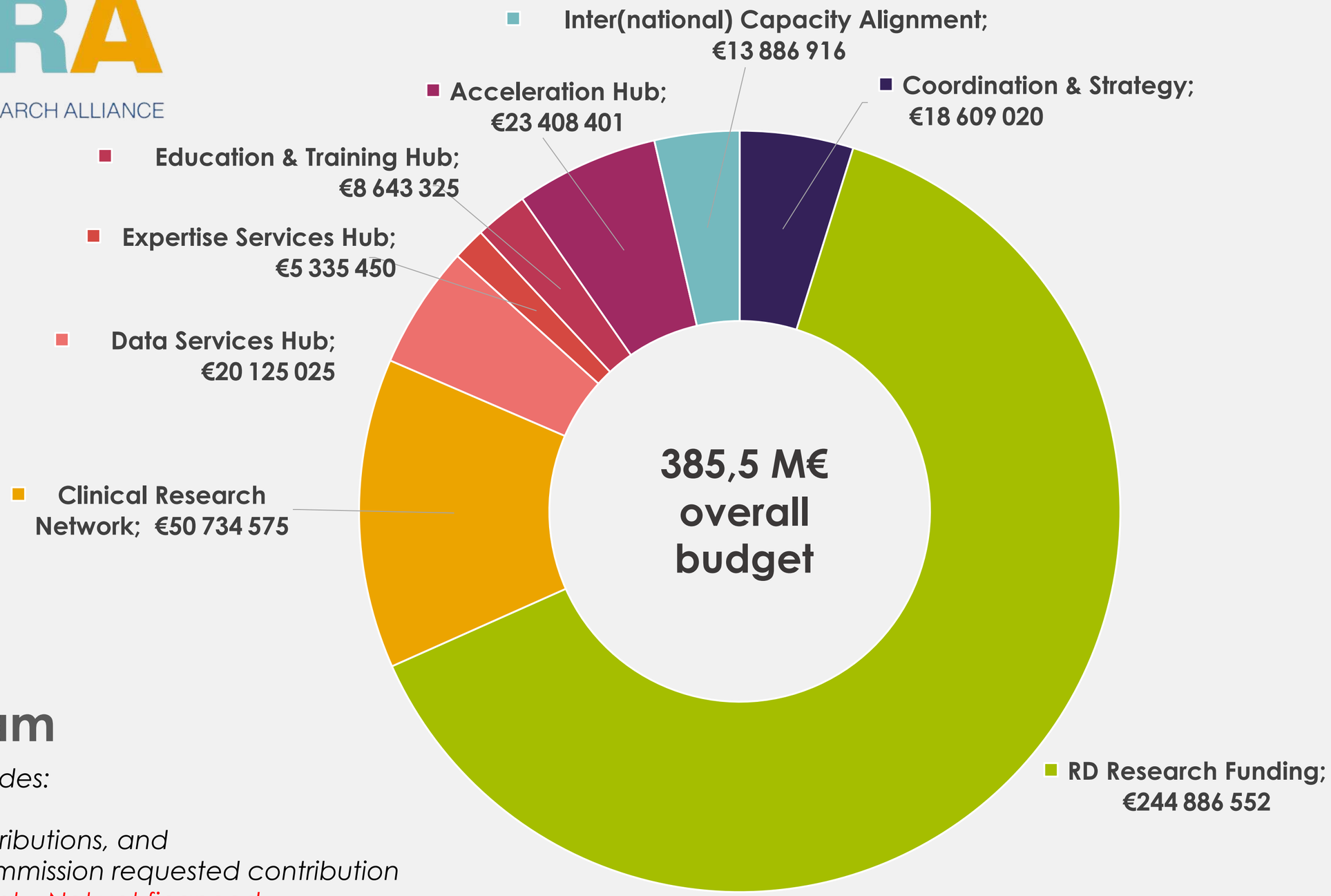

Total Budget* per Work Stream

**estimated for 7 years, includes:*
(1) associated partners,
(2) in-kind and in-cash contributions, and
(3) 160 M€ of European Commission requested contribution

Submitted Grant – Not yet financed

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EUROPEAN RARE DISEASES RESEARCH ALLIANCE

Coordination & Strategy

WP1
Coordination and management

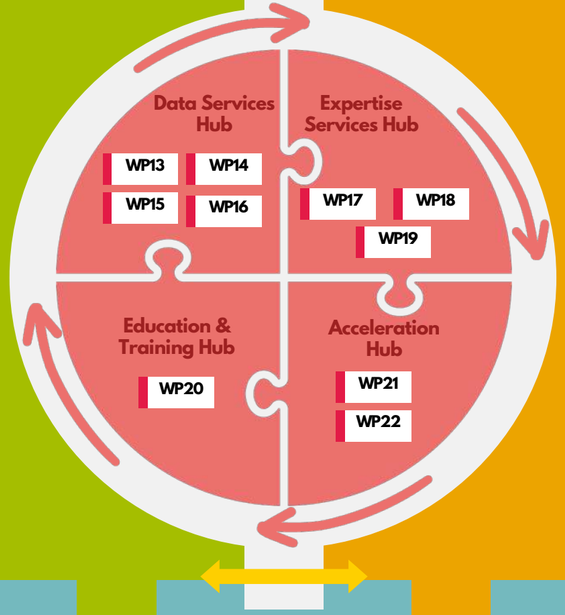
WP2
Communication & dissemination

RD Funding

WP3
Joint Transnational Calls for collaborative research projects

WP4
Clinical trial call management

WP5
Networking to share knowledge on research



Clinical Research Network

WP6
Diagnostic data availability

WP7
Genome re-analysis research pipeline

WP8
Innovation to shorten time to RD diagnosis

WP9
Real world data

WP10
Clinical Outcome Assessment

WP11
Advanced Therapeutic Medicinal Products

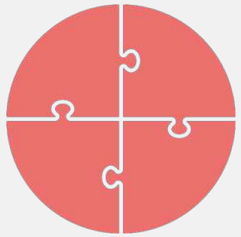
WP12
N-of-few approach

Inter(national) Capacity Alignment

WP23
NMGs promotion and national alignment

WP24
Fostering engagement of underrepresented countries in ERDERA

WP25
ERDERA Global Collaboration



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

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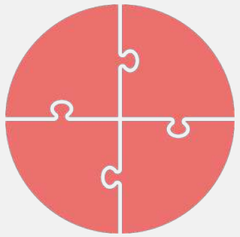

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Joint transnational calls for collaborative research projects

- **Tasks 3.1 –** Topics selection for the JTCs and Clinical Trial Call
- **Task 3.2 –** Joint Transnational Call implementation
- **Task 3.3 –** Working group on patient engagement in research project funding

Clinical trial call management

- **Tasks 4.1 –** Develop the call framework
- **Task 4.2 –** Open the call and select trials for funding
- **Task 4.3 –** Project implementation, project monitoring and financial management

Networking to share knowledge

- **Tasks 5.1 –** Preparation and launching of the funding scheme
- **Task 5.2 –** Evaluation of the selected proposals after each collection date
- **Task 5.3 –** Quality management

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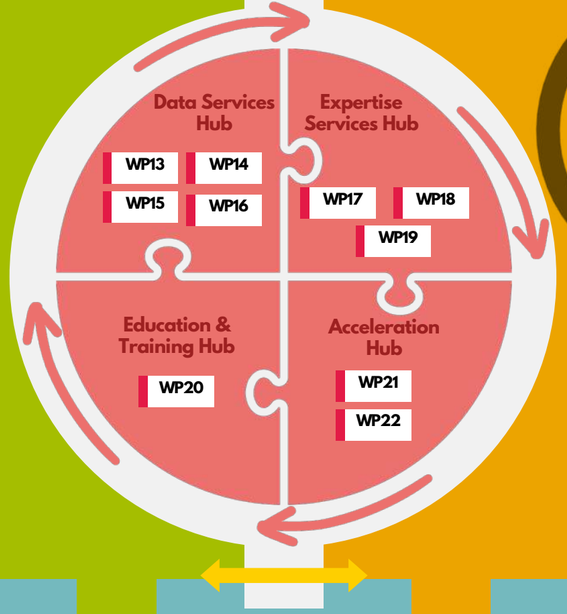

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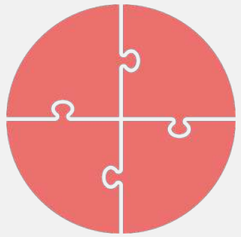

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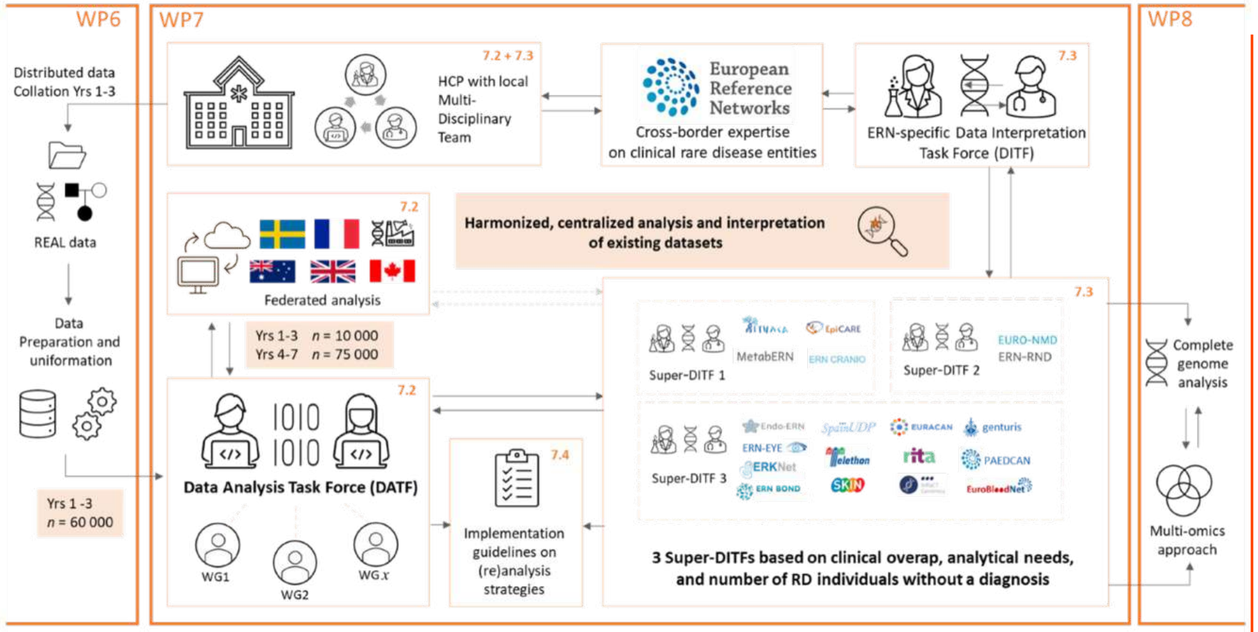
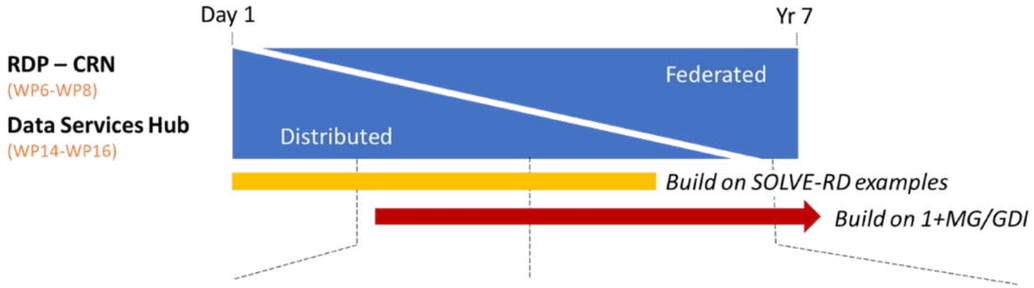
CRN: Diagnostic Research workstream

Diagnostics workflow



Moving towards federation



RDP – CRN
(WP6-WP8)

Data Services Hub
(WP14-WP16)

Accelerate diagnosis through Distributed approach (Phase I)
- → Upload phenotype + data
- → Coordinated analysis
- → Coordinated interpretation together with submitters for feedback to patients

For: local HCPs without infrastructure or bioinformatic support

Accelerate diagnosis through Distributed approach (Phase II)
- → Provide analytical tools & interpretation guidelines
- → Local analysis
- → Local interpretation
- → Share results with ERDERA

For: local HCPs without infrastructure or bioinformatic support

Accelerate diagnosis through Federated approach
- → Access to data in other centres
- → Local & federated interpretation
- → Share results with ERDERA

For: National programs and industry from the start, and allowing for scalability to local HCPs later in project

CRN: Analysis + interpretation incl. tool development
Data Hub: compute services and data platforms

CRN: Analytical + interpretation support incl. tool development
Data Hub: Operational/Deployment aspects
Intrastructure for Data Access: 1+MG / GDI

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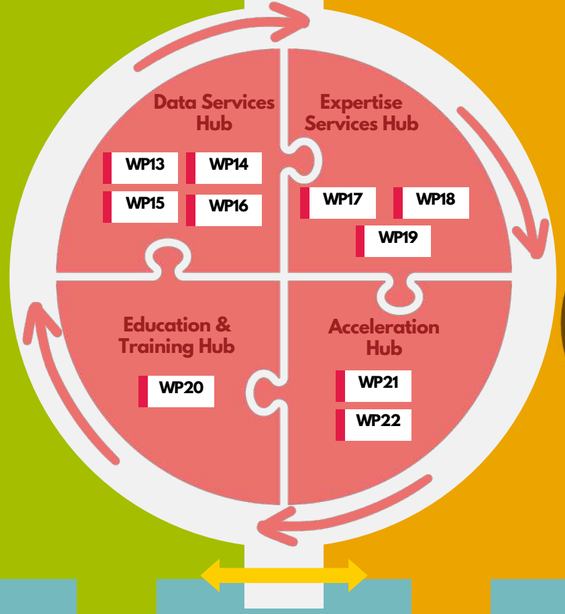

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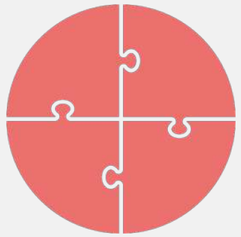

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CRN: Outcome Research workstream _ Involvement of ERNs

	9.1	9.2	9.3	9.4	9.5	9.6	10.1	10.2
BOND			x					
CRANIO	x						x	
ENDO			x					
EpiCare	x	x					x*	x
ERKNet			x	x		x		x
ERNICA		x						
EURO-NMD / DDF	x				x	x	x*	x
RND				x	x		x*	x
EuroBloodNet	x			x	x		x	x
eUROGEN	x							
EYE				x			x*	
ITHACA			x				x	
MetabERN		x					x*	

* Mito-InterERN workgroup

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CRN: Outcome Research workstream

Real World Data

- **Task 9.1 –** Use of primary healthcare data (EHRs) for RD outcome research
- **Task 9.2 –** Use of population-based data for RD outcome research
- **Task 9.3 –** Integration of patient cohorts for natural history / standard-of-care reference studies
- **Task 9.4 –** Blueprint and inventory of regulatory-grade natural history cohort data
- **Task 9.5 –** Disease progression modelling and prognostic biomarker research
- **Task 9.6 –** Development of a regulatory grade clinical trial simulation platform for rare diseases

CRN: Outcome Research workstream _ Real World Data

T9.1: Use of primary healthcare data (EHRs) for RD outcome research

Extraction of structured and unstructured data from electronic health records (EHRs) for registry/research re-use

- **Studied Disease Groups:**
  - Urogenital and craniofacial anomalies, neuromuscular disorders, encephalopathic epilepsies, mitochondrial diseases, rare anemias
- **Multi-Site Process:**
  - Involvement of EHRs from multiple **HCPs**, with various data **platforms** (commercial and internal) and different **languages**
- **Benchmarking:**
  - **Comparison of different solutions** on a common set of manually annotated EHRs in different languages
- **Automated Extraction:**
  - Demonstration of **automated EHR extraction** for each site.

CRN: Outcome Research workstream _ Real World Data

T9.1: Use of primary healthcare data (EHRs) for RD outcome research

Workflow:

Obtaining Institutional Approvals:

- Institutional approval and data use conditions for EHR data extraction

Data Pseudonymization:

- Data pseudonymization and determination of the level of anonymity

Data Compilation:

- Compilation of structured and/or unstructured data elements to be extracted

Ontological Mapping:

- Implementation of mappings to existing ontologies and semantic models, starting with "Common Data Elements"

Using NLP:

- Creation of structured output forms from unstructured texts using natural language processing (NLP) methods
  and **evaluation of their usefulness by clinical domain experts**

CRN: Outcome Research workstream _ Real World Data

T9.2: Use of population-based data for RD outcome research

Aim: Develop innovative approaches to strengthen population-based RD outcome research, including creation of minimal interoperative generic dataset for population-based studies, linking data from **European Reference Networks (ERN) and social security systems**

Three case studies to examine different care pathways: successive drug regimens, surgical, neonatal screening

Dravet Syndrome:

- Combines French National Rare Diseases Registry (**BNDMR**) and National Health Data System (**SNDS**) with **Italian national Dravet registry**
  to study **impact of drug therapies** on **life expectancy** and **health costs**

Hirschsprung's Disease:

- **Links national health insurance data** of patients with Hirschsprung's disease with **nationally collected outcome data** in **Belgium,**
  combined with equivalent national health data from the **BNDMR and SNDS in France**

Newborn Screening (NBS):

- Develops **generic neonatal screening module** with controlled vocabularies and ontologies,
  enabling **data preparation for AI/ML.** Tailored for **metabolic diseases** and implemented in the **U-IMD registry** of MetabERN.
- Aims to **empower evaluation of NBS programs across various diseases screened in EU countries**
  Interacts with multi-stakeholder platform **Screen4Rare/ERN** and European genomic NBS initiatives (**Screen4Care, NGS4NBS**)

CRN: Outcome Research workstream _ Real World Data

T9.3: Integration of patient cohorts for natural history / standard-of-care reference studies

Aim: To integrate natural history registry data into large, high-quality ("regulatory-grade") reference cohorts for future clinical trials, progression, QoL and intervention studies

- **"RASopathies":**
  - Merging of **three regional registries** from Italy, Germany, and France with **ILIAD** ERN registry and French national cohort
- **Steroid Resistant Nephrotic Syndrome (SRNS):**
  - Integration of **PodoNet** Registry SRNS cohort with sub-cohorts from **ERKReg** Registry, local (Paris) and national (UK) databases
- **Rare Endocrine (e.g. pituitary tumors) and Bone Disorders (e.g. Osteogenesis imperfecta):**
  - Consolidation of cohorts in EuRRECa/EURR-Bone registries with institutional and national databases
- **Hereditary Ataxias:**
  - Integration of **TreatHSP** (hereditary spastic paraplegia), **PROSPAX** (spastic ataxias), and **ARCA** (autos.rec. cerebellar ataxia) registries

FAIRification and Integration

- **All integrated cohorts** will be **FAIRified**, hosted on the **ERDERA Virtual Platform**, and **made available** in **clinical trial ready formats**.

Metadata Deposition and Future Integration

- Metadata from all cohorts will be deposited in **ERDRI**
- Concept for future integration into the **European Health Data Space (EHDS)** will be developed

CRN: Outcome Research workstream _ Real World Data

T9.4: Develop a blueprint and inventory of regulatory-grade natural history cohort data

Aim: Establish procedures to allow academic registries and cohort studies to obtain EMA qualification as external comparator arms in clinical trials, post-authorization safety monitoring, and efficacy studies

Development of a Procedural Model:

- Exemplary processing of European registries / cohort studies:
  mitochondrial disorders, hereditary spastic paraplegia, C3 glomerulopathy, retinal dysplasia

Creation of Inventory and Guide:

- Point-by-point inventory and guide will be developed, providing comprehensive support to registries/cohort studies
  aiming to achieve regulatory quality status

Collaboration with EMA:

- Collaboration with EMA's Data Analysis and Real World Interrogation Network (DARWIN-EU)
  Coordination with IHI consortium for innovative therapies for rare diseases

CRN: Outcome Research workstream _ Real World Data

T9.5: Disease progression modelling and prognostic biomarker research

Aim: To develop and apply innovative methodologies for disease progression modeling in RDs

Spastic Ataxias (SPAX):

- Use of longitudinal multimodal datasets established in task 9.3
- **Innovative Statistical Toolkit** for predicting individual/group disease progression based on clinical profiles and biomarkers
- **Simulation of treatment-induced variations** to set stage for innovative n-of-1 and n-of-few therapeutic trials (WP12)

Sickle Cell Disease:

- Risk modeling to improve disease characterization and health status monitoring, personalize therapeutic approaches, optimize health outcomes
- **Multimodal Data Analysis**: metabolomic, genomic, and radiomic data

Spinal Muscular Atrophy:

- SMArtCARE registry: longitudinal data from patients treated with one of the three approved drugs
- Model SMA disease trajectories and test reproducibility of clinical trial outcomes in real-world context

Methodological Support:

- ERDERA's modeling expert group (WP19)
- Extension of EJP-RD work on individual disease prediction, uncertainty quantification, simulation of treatment-induced variations

CRN: Outcome Research workstream _ Real World Data

T9.6: Development of a model-based clinical trial simulation platform for RDs

Aim: Demonstrate development of **Drug Development Tools (DDTs)** that will improve understanding of RD progression patterns
and help predict more rapidly, with greater certainty and at lower cost, the efficacy and safety of new drugs

Prediction Models:

- Combine natural history data and data from previous clinical trials to develop prediction models that can simulate the effects of therapeutic interventions on clinical endpoints and estimate required number of subjects, using conventional trial methodologies and small population approaches

Data Modelling: on C-Path RDCA-DAP platform

Formal Review and Regulatory Endorsement:

- Develop strategy for formal review and potential regulatory approval of disease progression models based on quantitative methods and/or artificial intelligence as DDTs

Therapeutic Areas (selected based on their advanced stage for regulatory qualification and initial modelling experience):

- Duchenne Muscular Dystrophy (DMD)
- Autosomal Dominant Polycystic Kidney Disease (ADPKD)

CRN: Outcome Research workstream _ Real World Data

T9.6: Development of a model-based clinical trial simulation platform for RDs

Transcontinental Collaboration:

- For ADPKD: ERKNet registry cohort of over 3,000 patients, C-Path, and US-based PKD Outcome Consortium will undertake transcontinental collaboration to develop a globally applicable PKD progression model towards regulatory maturity as a DDT.

Modelling Expertise:

- The experience of the Duchenne Data Foundation in innovative machine learning methods and C-Path's expertise in developing quantitative solutions will be combined to generate an enhanced Clinical Trial Simulation (CTS) tool for DMD.

Extension to other Diseases:

- Modelling expertise generated in ADPKD project for modelling loss of glomerular function (eGFR) will also be considered for other progressive rare kidney diseases with a therapeutic perspective for which high-quality Real-World Data is available.

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CRN: Outcome Research workstream

Clinical Outcome Assessment

Task 10.1 – Platform for development and validation of regulatory-grade patient-centred COA

Task 10.2 – Development and Implementation of Clinical Outcome Assessment Tools

Task 10.3 – Unveiling the Hidden Burden: Estimating the Socioeconomic Impact of Rare Diseases for Informed Decision Making and Resource Allocation

CRN: Outcome Research workstream _ Clinical Outcome Assessment

T10.1: Platform for development and validation of regulatory-grade patient-centred COA

Patient-centered multi-stakeholder process to develop patient-reported outcome (PRO) assessment tools and evaluate patient relevance of top-ranked clinical outcome assessment (COA) tools for five rare disease groups: HSP/ataxia [RND], RASopathies [Ithaca], rare anemia disorders [EuroBloodNet], craniofacial anomalies [CRANIO], mitochondrial diseases [5 ERNs]

- **Establishing Reference Data Sets Coded According to International Classification of Functioning (ICF)**

- **Matching with Existing PROMs**
  Match ICF-coded functional impacts with appropriate existing patient-reported outcome measures (PROMs)
  (Collaboration with ERICA)

- **Validation of PROMs in at least three European languages**
  Establish sensitivity to change, meaningful change to the patient, and equivalence between languages

- **Anchoring Studies for COA Candidates:**
  Establish relevance of top-ranked COA candidates to patients

T10.2: Development and Implementation of Clinical Outcome Assessment Tools

Development of methodologies to collect and implement data from Patient-Centered Outcome Measures (PCOMs) related to patient registries, considering legal, technological, and regulatory requirements, and validation in 4 case studies using mobile health solutions and devices for rare neurological, neuromuscular, renal, and hematological disorders.

- **Study Protocol Development:** Collect clinical and technical requirements from end-users and standardize data (Collaboration with WP13)
- **Identification of Mobile Apps and Devices** and assessment of their research readiness (Collaboration with WP14)
- **Clarification of Regulatory Aspects,** including privacy and security protections in mobile health (Collaboration with WP18)
- **Development/Adaptation of Mobile Health Solutions** for use with patient registries (Collaboration with ERN Registries)
- **Execution of Clinical Studies**
- **Validation of Patient-Generated Data**
- **Development of a Data Visualization Framework**

T10.3: Unveiling the Hidden Burden: Estimating the Socioeconomic Impact of RDs for Informed Decision Making and Resource Allocation

Implementation of a framework to estimate the socioeconomic impact of Rare Diseases (RD) in Europe based on Real World Data through the mapping of data, the calculation of Disability Adjusted Life Years (DALY), and the estimation of costs in ten RDs and their associated registries.

- **Protocol for DALY Calculation:** A protocol based on harmonized and improved methods to calculate the DALYs of RDs. It will include consensus disease models, describing the main health states and disability weights.
- **Protocol for Disease Cost Calculation:** A protocol based on harmonized and improved methods to calculate the Cost of Illness (COI) associated with RDs, including caregivers. This includes the assessment of direct medical costs and the evaluation of indirect costs by monetizing the Years of Life Lost (YLL).
- **Visualization of Impact:** The protocols will include methods for visualizing the impact of different scenarios, for example, the impact of a new treatment on DALY and COI estimates.
- **Stakeholder Strategy:** A dedicated stakeholder strategy targeting patient registry owners will promote adoption at the national level, aiming for routine quantification of the socioeconomic impact of RDs.

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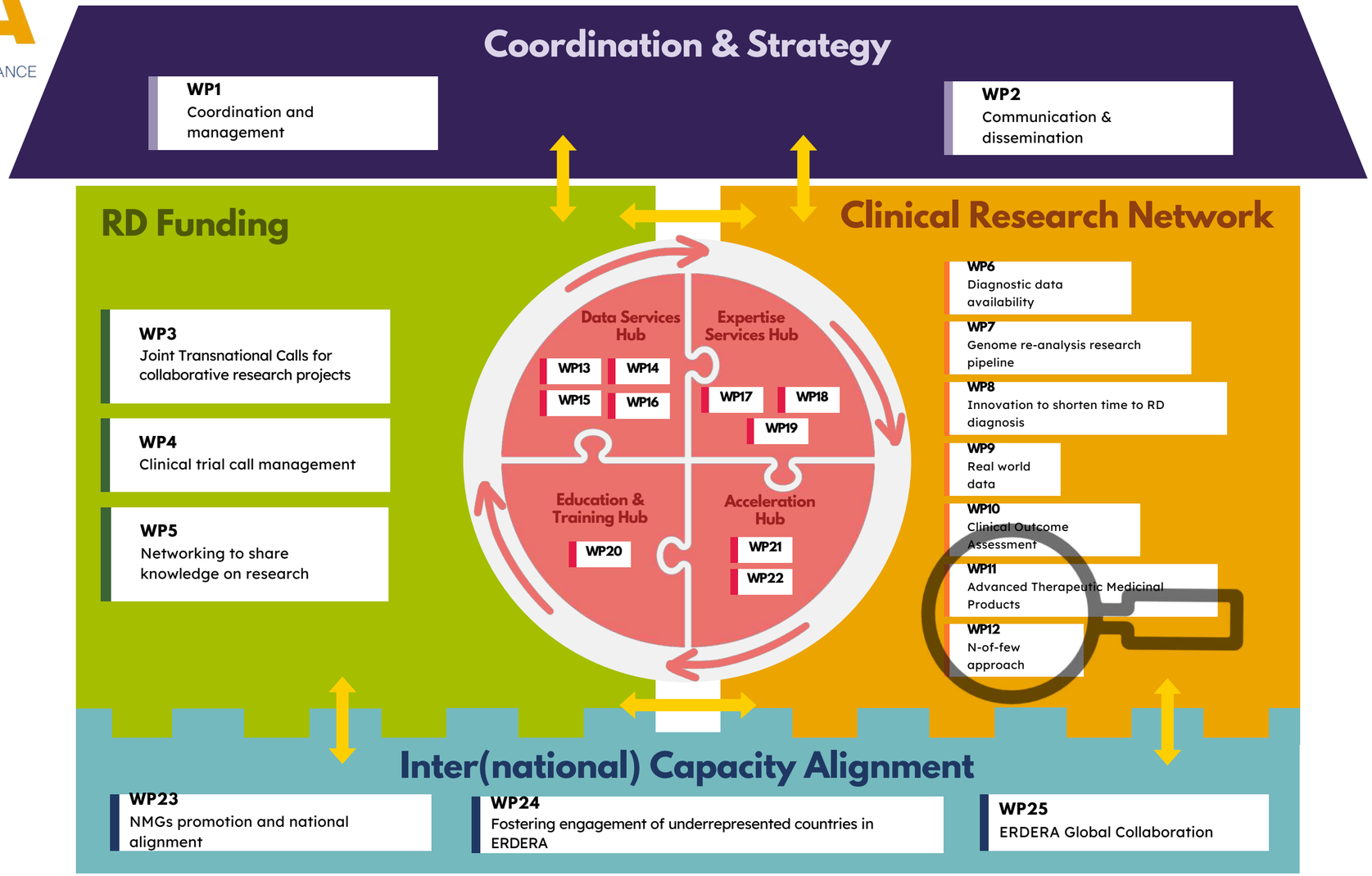

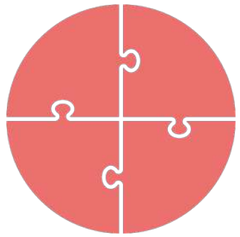

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CLINICAL RESEARCH NETWORK _ Innovative therapies

Advanced Therapeutic Medicinal Products

- **Task 11.1** – Identify and rank disease indications requiring ATMPs
- **Task 11.2** – Match technical development with prioritised needs
- **Task 11.3** – Elaborate PoC studies to test the development pipeline
- **Task 11.4** – Evaluate the selected platforms for clinical trials requirement and joint transnational call

N-of-few approach

- **12.1** – Academic Platform for Tailored Antisense Oligonucleotide Therapies
- **12.2** – Identification of patient relevant-outcomes (n-of-1/few) and run in natural history study
- **12.1** – Treatment/study design and analysis
- **12.1** – Implementation of first in human treatment infrastructure
- **12.1** – Case studies

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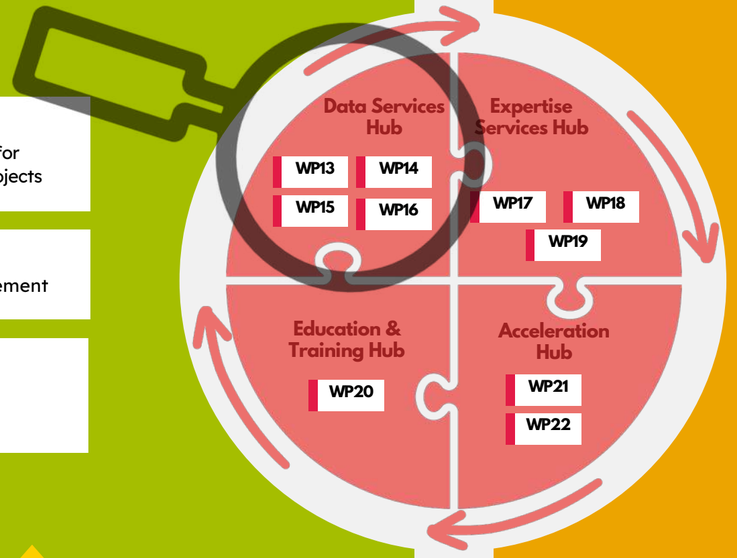

Inter(national) Capacity Alignment

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NMGs promotion and national alignment

WP24
Fostering engagement of underrepresented countries in ERDERA

WP25
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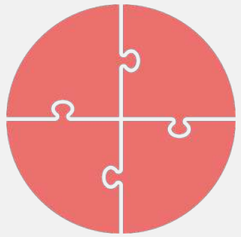

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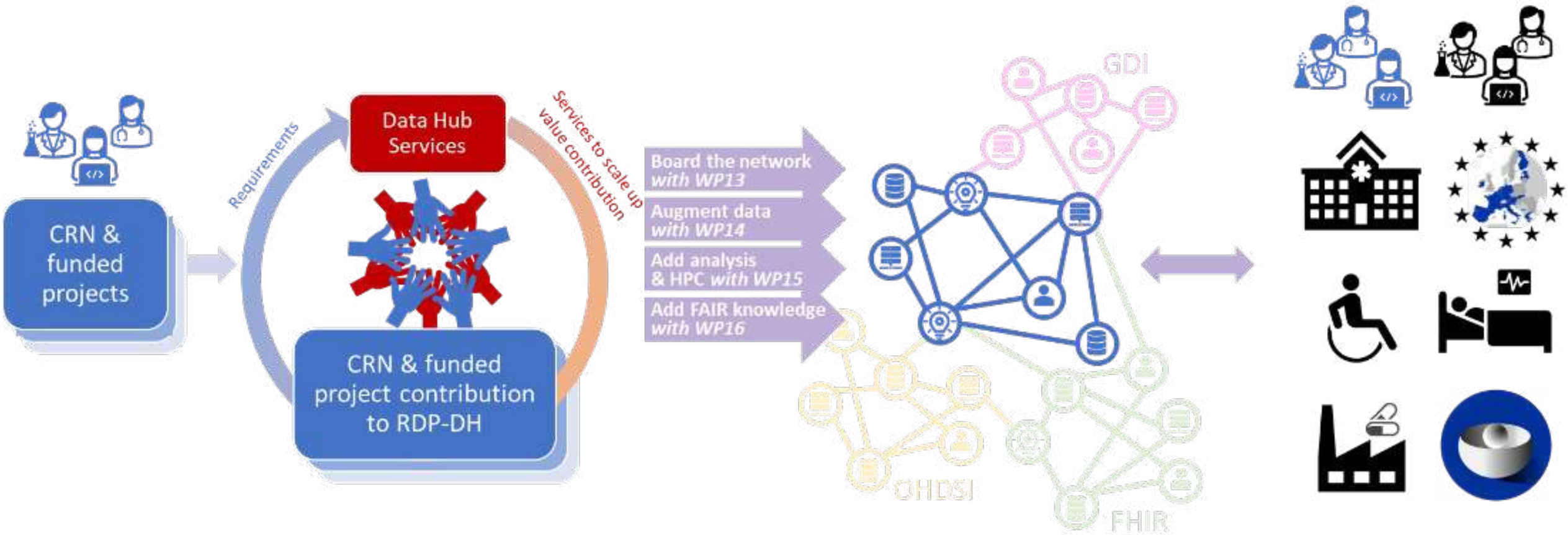
Data Services Hub



Co-creating value in partnership

RDP Data Hub as value proposition
Data, Analysis,
Computing infrastructure, Knowledge

Accelerate outcomes for RDs

Co-creating the ERDERA Data Hub as value proposition

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EUROPEAN RARE DISEASES RESEARCH ALLIANCE

Data Services Hub

WP13

RD-VP: finding & accessing data ecosystem

- 13.1 – Steering of the data service hub (DSH) and the RD-VP
- 13.2 – RD VP network evolution and scaling up
- 13.3 – VP onboarding services

WP14

Data readiness services

- 14.1 – Services for making data FAIR for automated applications
- 14.2 – Data ingestion services
- 14.3 – Services to prepare data for the regulatory pathway

WP15

Data sharing and analysis services

- 15.1 – Data archiving and sharing services
- 15.2 – Data analysis infrastructure as a service
- 15.3 – Federated analysis

WP16

Knowledge bases, ontologies & semantic models for RD research

- 16.1 – Creation of a repository of FAIR PCOMs/ PROMs
- 16.2 - Antenatal echographic and pathologic RD phenotypes knowledge base and ontology
- 16.3 – Treatabolome
- 16.4 – Rare disease maps
- 16.5 – Improving and expanding RD Ontologies and semantic models: ORDO,HOOM,HPO, functional impact of RD

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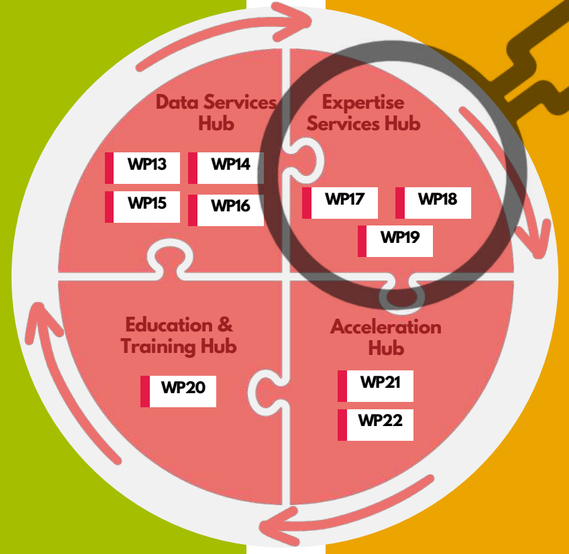

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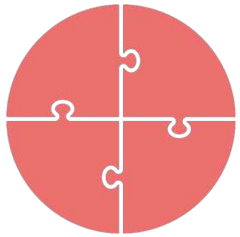

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Expertise Services Hub

Mentoring and Consultancy

- **Task 17.1 -** Execution of the Mentoring Program
- **Task 17.2 -** Consultancy Services

Regulatory Support service

- **Task 18.1 -** Regulatory support to preclinical research
- **Task 18.2 –** Regulatory support to clinical research

Methodological Support

- **Task 19.1 -** Knowledge transfer towards the local clinical trial teams
- **Task 19.2 -** Novel methodologies for the use of all available knowledge, including Real World Data
- **Task 19.3 –** Data analysis methodologies when data are multivariate, hierarchical, incomplete and of differing data types
- **Task 19.4 -** Non-parametric and randomized-based methodology

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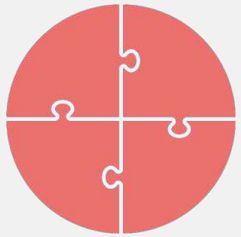

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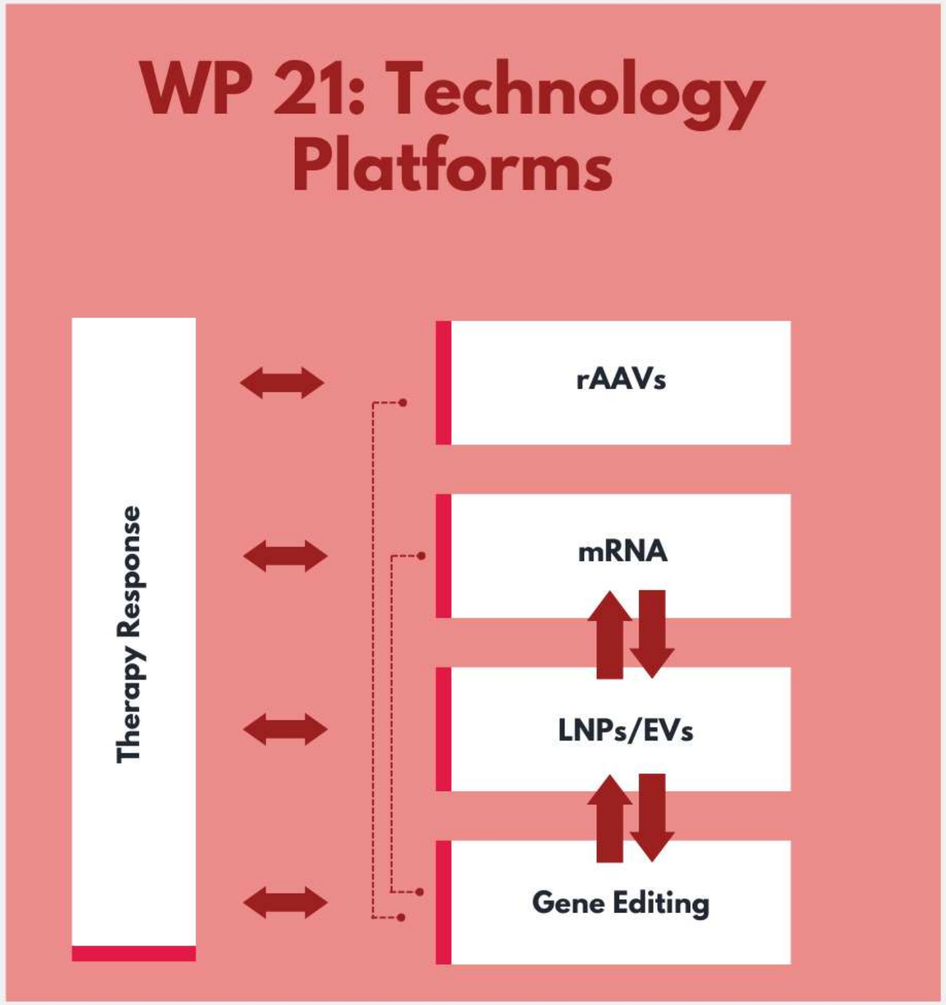

Innovative ATMP technologies	WP21
Regulatory support	WP19
Standardization guidelines	WP19
Connection with clinical networks	WP11
Patient engagement	WP1
Data infrastructure	DSH Data Services Hub
Policy guidelines	WP1

rAAV: recombinant Adeno-Associated Virus
LNPs: Lipid Nanoparticles
EVs: Extracellular Vesicles
DSH: Data Services Hub

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Acceleration Hub

Innovative therapies technology accelerator

- **Task 21.1** – Streamlining chemistry manufacturing and controls in AAV vector production
- **Task 21.2** – Advancing RNAs for therapeutics
- **Task 21.3** – Advancing of novel types of mRNA nanoparticles (NP) including Lipid NP, extracellular vesicles and biohybrids
- **Task 21.4** – Gene editing approaches towards therapy of rare diseases
- **Task 21.5** – Assessment of therapy response and immunogenicity

Public-Private collaboration accelerator

- **Task 22.1** – Setting up the marketplace
- **Task 22.2** – Acceleration readiness
- **Task 22.3** – Asset profiling and development
- **Task 22.4** – Matchmaking and marketing

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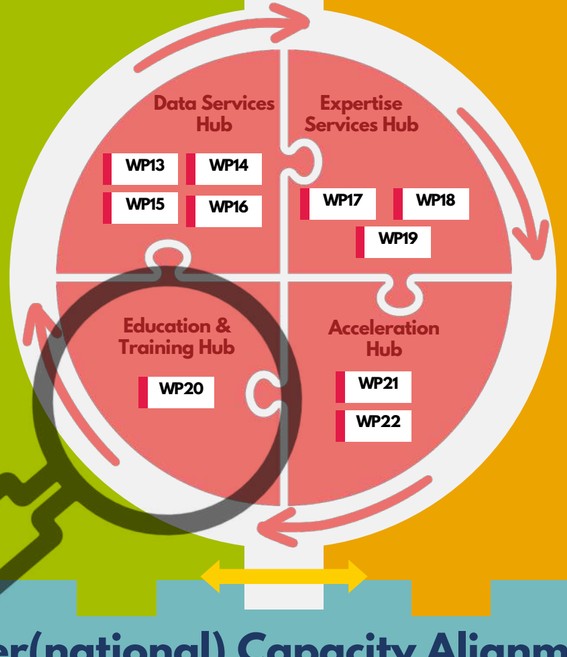

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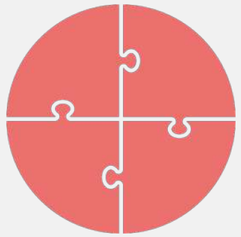

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Education and training in rare diseases research

Patients and young researchers' trainings

Blended and scalable training programme for patients across the whole RD research pipeline

3 Levels: Beginner, specialized pathways, Advanced
To be started during year 1 *(each step will be held once each)*

Training workshops for pediatric patients

Focus on the following areas:
(1) rare and genetic diseases specificities;
(2) patients' and children rights;
(3) clinical and translational research challenges for paediatric patients and relevance of their role (ethical and legal issues);
(4) patients' engagement in CTs, participation in CTs and other studies (such as innovative, PROMs, PREMs, etc.); the consent/assent issues, innovative therapies in the context of paediatric RDs, paediatric lay summaries, etc);
5) Orphan Medicinal Products availability and access for paediatric patients on the EU market, pharmacovigilance and off-label use in paediatric setting
To be started during Year 1: *a 2-to-3-day training course, targeting up to 15-20 young RD patients (12-18 years old).*

Education and training in rare diseases research

RD research trainings for multistakeholder community

Trainings to empower RD diagnostic research

To be started during Year 1: *preparatory online lectures, webinars, and modules will be prepared for two thematic training groups: a) Management of genomic and clinical data for re-analysis and b) Introduction to innovative omics.*

Trainings to empower RD clinical research

To be started during Year 1: *Training "Cell & gene therapy development"*

Trainings to empower knowledge on research methodologies

To be started during Year 1: *Training "Best practice for successful iPSC research"*

Trainings to empower acceleration of research and RD clinical trial methodologies and management

To be started during Year1: *Trainings "Evidence synthesis for clinical studies" and "Management of multinational trials"*

Trainings to empower data research

To be started during Year 1: *scaled approach of gradual development of trainings is foreseen from beginner-level*

Education and training in rare diseases research

European Curriculum on RD research

Aim: establish a higher education program culminating in an academic diploma with credits, through an inter-university consortium of academic and non-academic partners with track record in RD training activities. T20.4.

1-Establish a cross ERDERA expert consortium and setup of training modules (M1-M12). Multilateral agreements will be made among academic and non-academic partners and connections with European university alliances, to ensure policy alignment and improve geographical representation as well as to ensure the smooth integration of young RD researchers into the EU R&I ecosystem

2-Pilot program launch (M13-24). This will run as a short-time pilot program (min 10 ECTS).

3-Optimization and scale-up of the program (M25-36). The study plan will be refined and potentially upgraded to longer-term education programs to address emerging needs and provide recognized diplomas.

4-Running the first systematic EU diploma curriculum (M30-60). Enrolling the first cohort of students, the curriculum will include a transnational mobility schedule and teaching modules from different partners.

5-Optimization and updating of study plan (M52-M84). Continuing adaptation of the course.

6-Pursue future sustainability of the established curricula (M6-84). Collaboration with stakeholders will be established to secure the program's sustainability (incl. opportunities offered through Erasmus Mundus programme).

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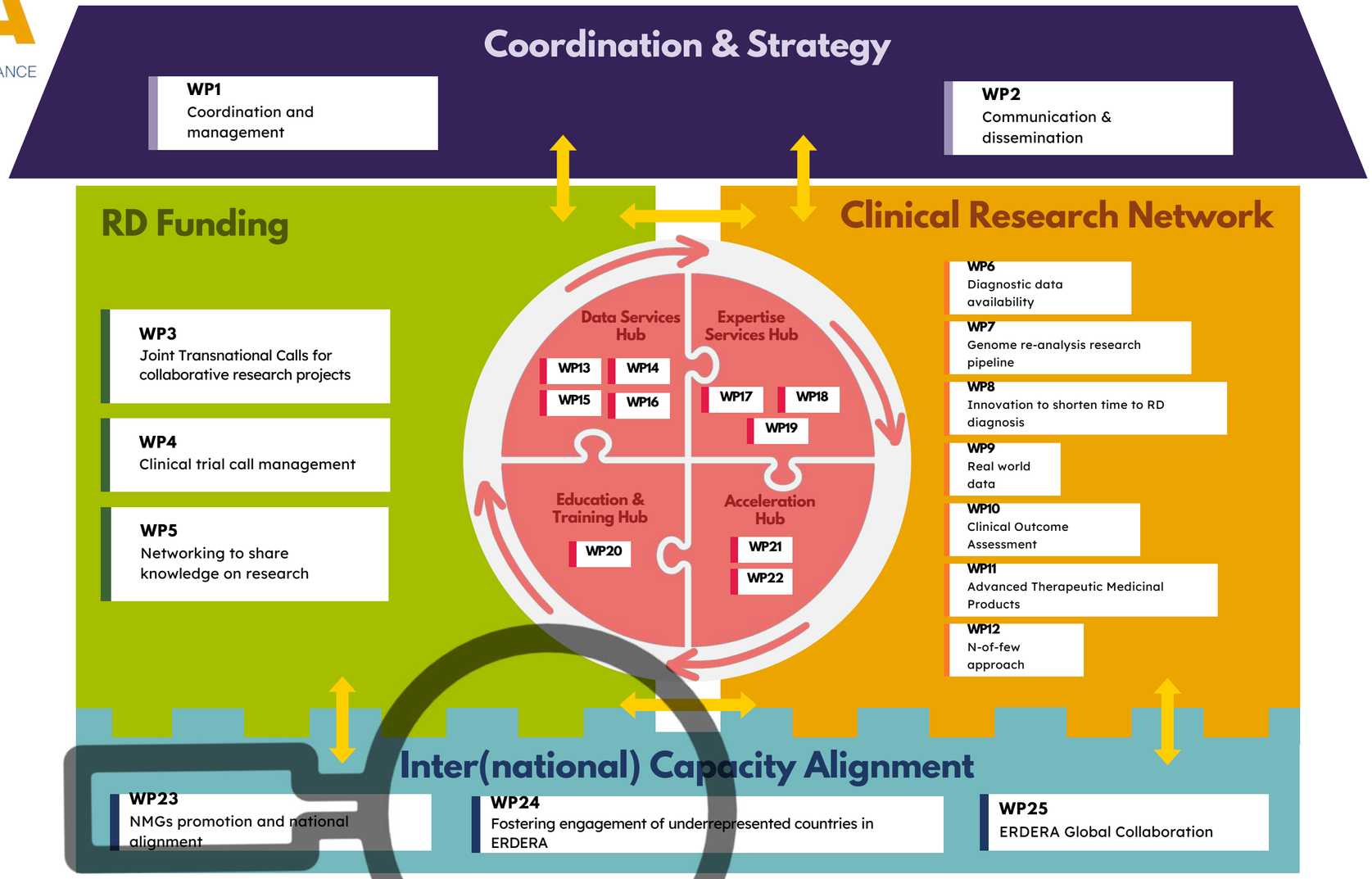

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(Inter)national capacity alignment

National Mirror Groups promotion & national alignment

- **Task 23.1** – Fostering creation of National Mirror Groups
- **Task 23.2** – Deployment and operations of National Mirror Groups
- **Task 23.3** – Animation of National Mirror Groups synergies

Fostering engagement of underrepresented countries

- **Task 24.1** – Promoting capacity development actions
- **Task 24.2** – Undertaking advocacy and awareness efforts to Underrepresented Countries added value
- **Task 24.3** – Support actions to improve Underrepresented Countries participation in all ERDERA activities

ERDERA Global collaboration

- **Task 25.1** – Strategic Alliances
- **Task 25.2** – IRDiRC Scientific Secretariat
- **Task 25.3** – Promote the International Dimension of the CRN of ERDERA by building global networks among CRNs and patient organizations.
- **Task 25.4** – Alignment with the Research strategies of the European Reference Networks

FUNDING

ERA4Health Partnership

PerMed

European Directory of Registries (ERDRI.dor)

Central Metadata Repository (ERDRI.mdr)

ERDRI

Search Broker (ERDRI.sebro)

SPIDER

EOSC

The European Open Science Cloud

EOSC-Life

DATA SERVICE HUB

Global Alliance for Genomics & Health

Collaborate. Innovate. Accelerate.

DARWIN EU

ACCELERATION HUB

INNOVATIVE SME

innovative health initiative

EHDS

HealthData@EU Pilot

JARDIN

European Genomic Data Infrastructure

RARE DISEASE MOONSHOT

SCALING UP PUBLIC-PRIVATE PARTNERSHIPS TO ACCELERATE RESEARCH IN RARE DISEASES

eit Health

THCS

TRANSFORMING HEALTH AND CARE SYSTEMS

SCREEN 4CARE

EDUCATION & TRAINING

CLINICAL RESEARCH NETWORK

Thank you

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