



ERICA

European Rare Disease Research
Coordination and Support Action



Endo-ERN

European Reference Network
on Rare Endocrine Conditions



European
Reference
Networks

#ERNcare4Ua

Rare Diseases Doctors

Strengthen research and innovation capacity by the integration of ERN research activities



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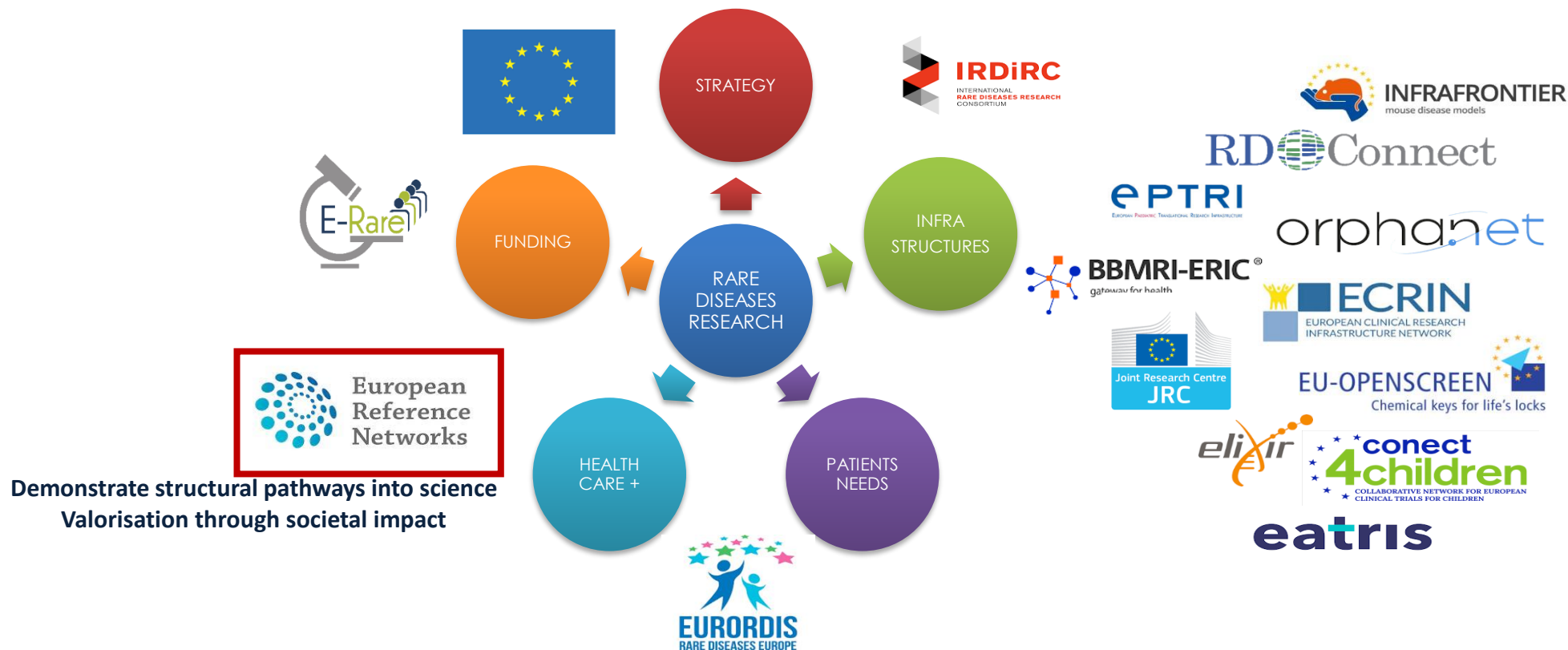


ERICA has received funding from the European Union's Horizon 2020
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European
Reference
Networks

Rare Diseases Landscape in Europe



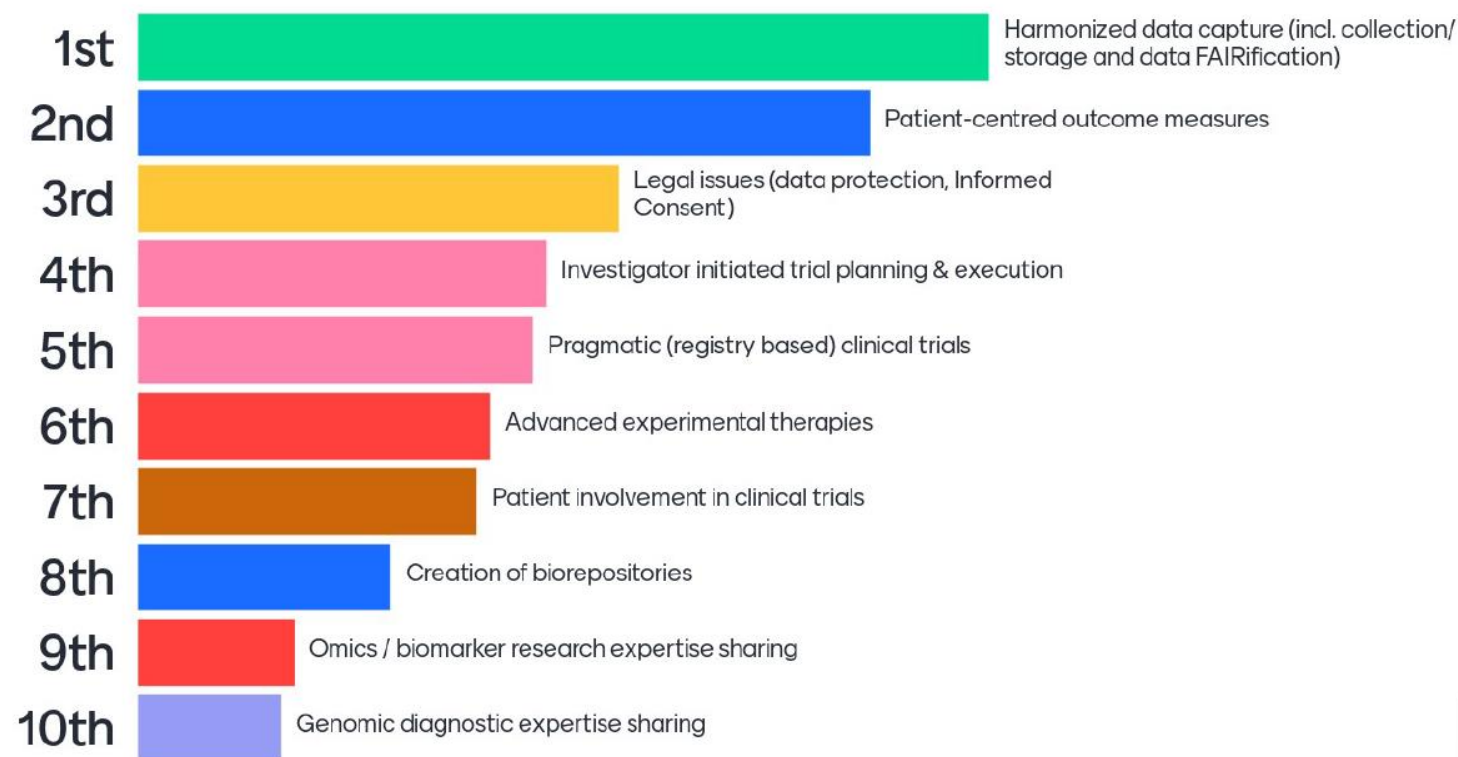


Aim: to strengthen ERN's research and innovation capacity, through:

- facilitating collaboration between ERNs
- increase the visibility and impact of ERNs (outreach and collaboration with multiple stakeholders)
- Optimal integration of the results into the new European Rare Disease Alliance (ERDERA) 2024-2030



What do you think are the most eminent topics for ERN research (rank at least 3 top)



WP2
Data Collection,
Integration and
Sharing

WP3
Patient-Centred
Research

WP4
Clinical Trial Support

WP5
Translation and
Innovation

WP6
Integration, Outreach
& Dissemination

Goals of ERICA

- effective data collection strategies
- better patient involvement
- enhanced quality and impact of clinical trials
- increased awareness of ERNs innovation potential
- new intra- and inter-ERN rare disease competitive networks



ERN registries: platforms integrating care & research:

- Registries, by enabling surveillance, audit, and research through a virtual environment have the clear potential to improve the care of people with rare and complex conditions
- Registries play a vital role in benchmarking clinical outcomes and act as a platform for quality improvement
- All ERNs: already > 50,000 included patients with RD!



Towards European Health Data
Space



Examples



WP2

**Data Collection,
Integration and Sharing**

WP2
Data Collection,
Integration and
Sharing



- Establishment of common guidelines for collecting, reusing and sharing data across ERNs and with other different stakeholders
- Encourage interoperability and standardisation

Data capture as starting point:

- Definition of data elements tied to registry objectives
- Clearly defined Data elements to ensure consistency in interpretation across participating sites
- Need to balance collected elements with the burden associated with data collection

Data reuse and access



- Standardized, GDPR compliant, machine readable Informed Consent Form in all EU languages.



- Standardized Collaboration / Data Transfer / Data Sharing Agreements



- Harmonized Data Access Policy



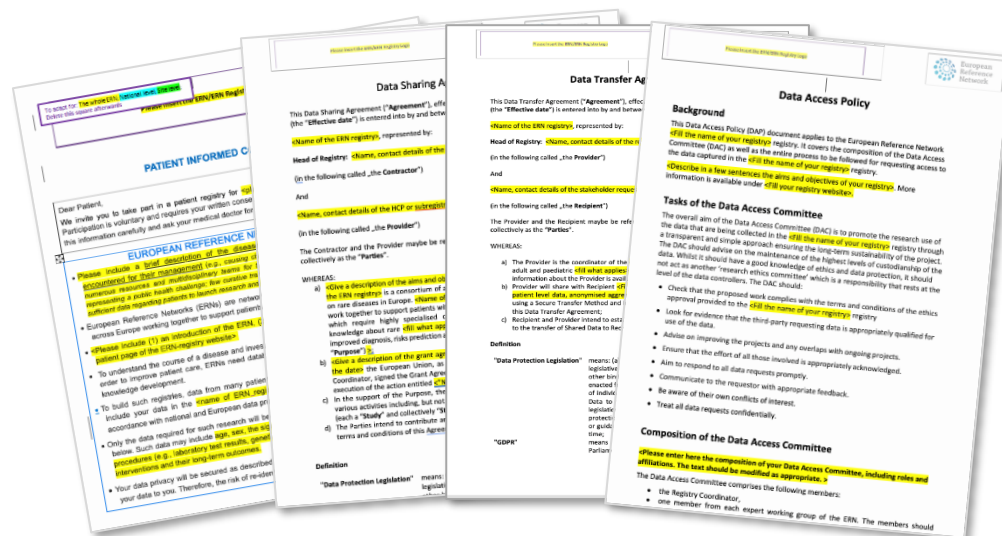
Issues when sharing aggregated data:

- Different legal frameworks
- Sufficiently anonymised?
- Does it qualify as personal or non-personal data?



Establish common guidelines for ERNs:


- Review applicable regulations
- Methods to prevent re-identification
- Discussion of obfuscation techniques



ERN Clinical Trials Repository

Filters list of Clinical Trials

Name of Study ¹⁾	<input type="text"/>	ERNs ²⁾	<input type="text"/>
Type ³⁾	<input type="text"/>	Status ⁴⁾	<input type="text"/>
OrphaCode ⁵⁾	<input type="text"/>	Disease ⁶⁾	<input type="text"/>
Phase ⁷⁾	<input type="text"/>	Begin Date ⁸⁾	<input type="text"/>



 Legend

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PROMs Repository

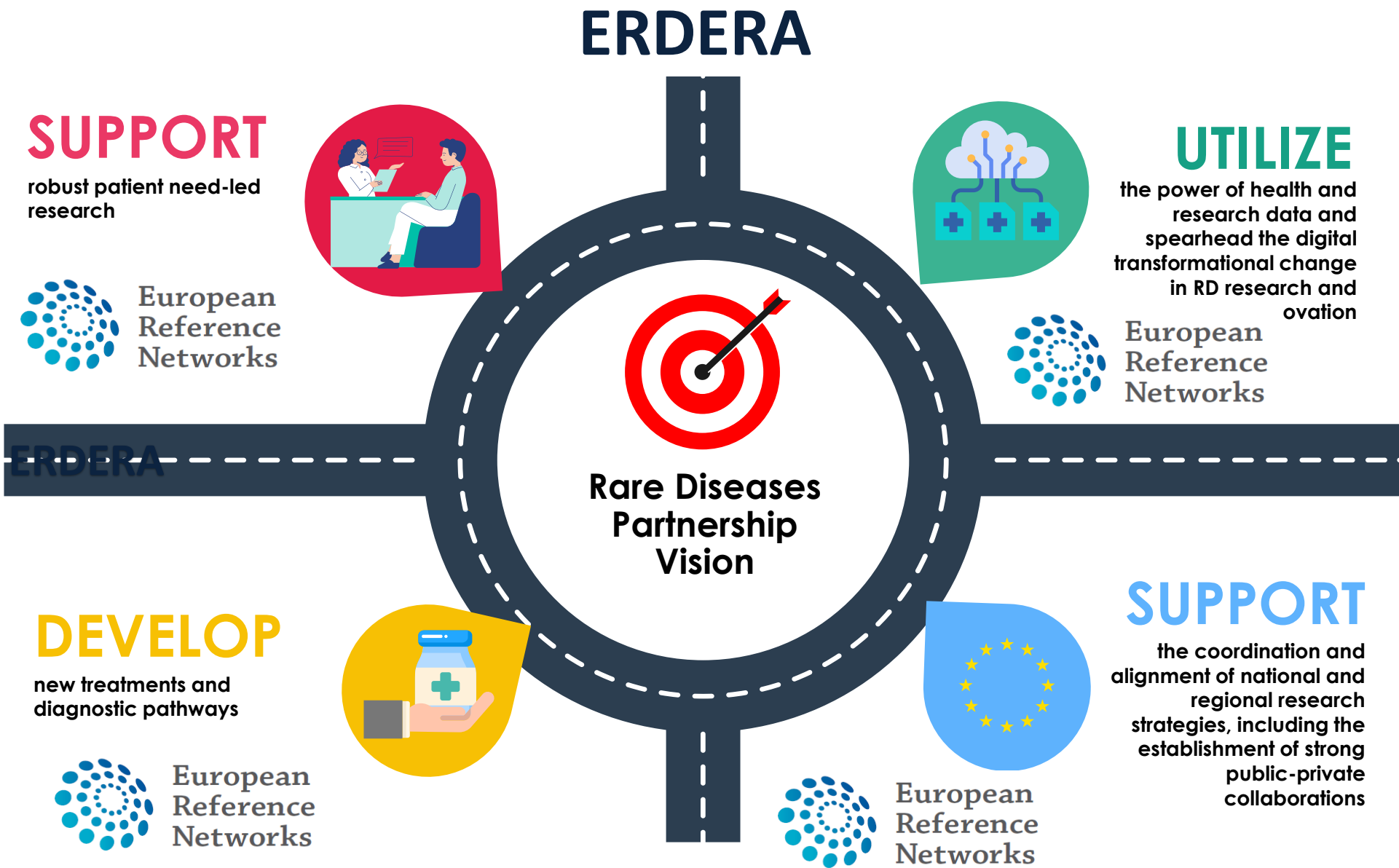
The ERICA Patient Reported Outcome Measures (PROMs) Repository is the first attempt to identify and centralize Clinical Assessment Outcomes questionnaires of relevance for rare diseases and constitutes a milestone in the Europe-wide standardization of Patient-Centered Outcome Measures (PCOMs) and PROMs for rare diseases. It has been made possible through the joint collaboration between **Orphanet**, **Mapi Research Trust/ICON** and **ERN EuroBloodNet** (VHIR, APHP), and the active contribution of ERNs and ePAGs. The methodology for the constitution and future evolution of the repository can be found in **deliverable 3.1**  849 KB) and **deliverable 3.2**  661 KB).

The central repository is a dynamic and evolutive service and should be regarded as a centralized and standardized access gate to more in depth information contained in [PROQOLID™](#).

Filters list of PCOMs/PROMs

PCOM/PROM Name ¹⁾	<input type="text"/>	PCOM/PROM Type ²⁾	<input type="text"/>
Target Age ⁴⁾	<input type="text"/>	Domains ⁵⁾	<input type="text"/>
Disease (OrphaName) ⁶⁾	<input type="text"/>	OrphaCode ⁷⁾	<input type="text"/>
Group of Diseases ⁸⁾	<input type="text"/>	ERNs ⁹⁾	<input type="text"/>

 Legend



‘Strengthen research and innovation capacity by the integration of ERN research activities’

- ERNs already have shown to harbour great research and innovation capacity
- The ERNs are able to deliver on all core activities, and clearly demonstrate structural pathways into science
 - ERNs translation and innovation: Development of new treatments and diagnostic pathways
 - ERN registries provide a unique European platform linking real world data on excellence in complex care to research.
- ERICA and the ERNs are projects, very successful ‘start-ups’ that now need to mature to professional organisations for 30 M patients with RD in the EU

The rare disease community comes together in calling on the EU institutions and our national governments to stand by the European Reference Networks.

Dear Mrs von der Leyen,
President of the European Commission,

Dear Mrs Metsola,
President of the European Parliament,

Dear Heads of Governments of EU Member States,

