



European
Reference
Network

for rare or low prevalence
complex diseases

 Network
Neuromuscular
Diseases (ERN EURO-NMD)

7th ERN EURO-NMD ANNUAL MEETING

ERICA : the European Rare disease research
Coordination and support Action

21st – 23rd February 2024

Ana Rath, on behalf of the ERICA
consortium

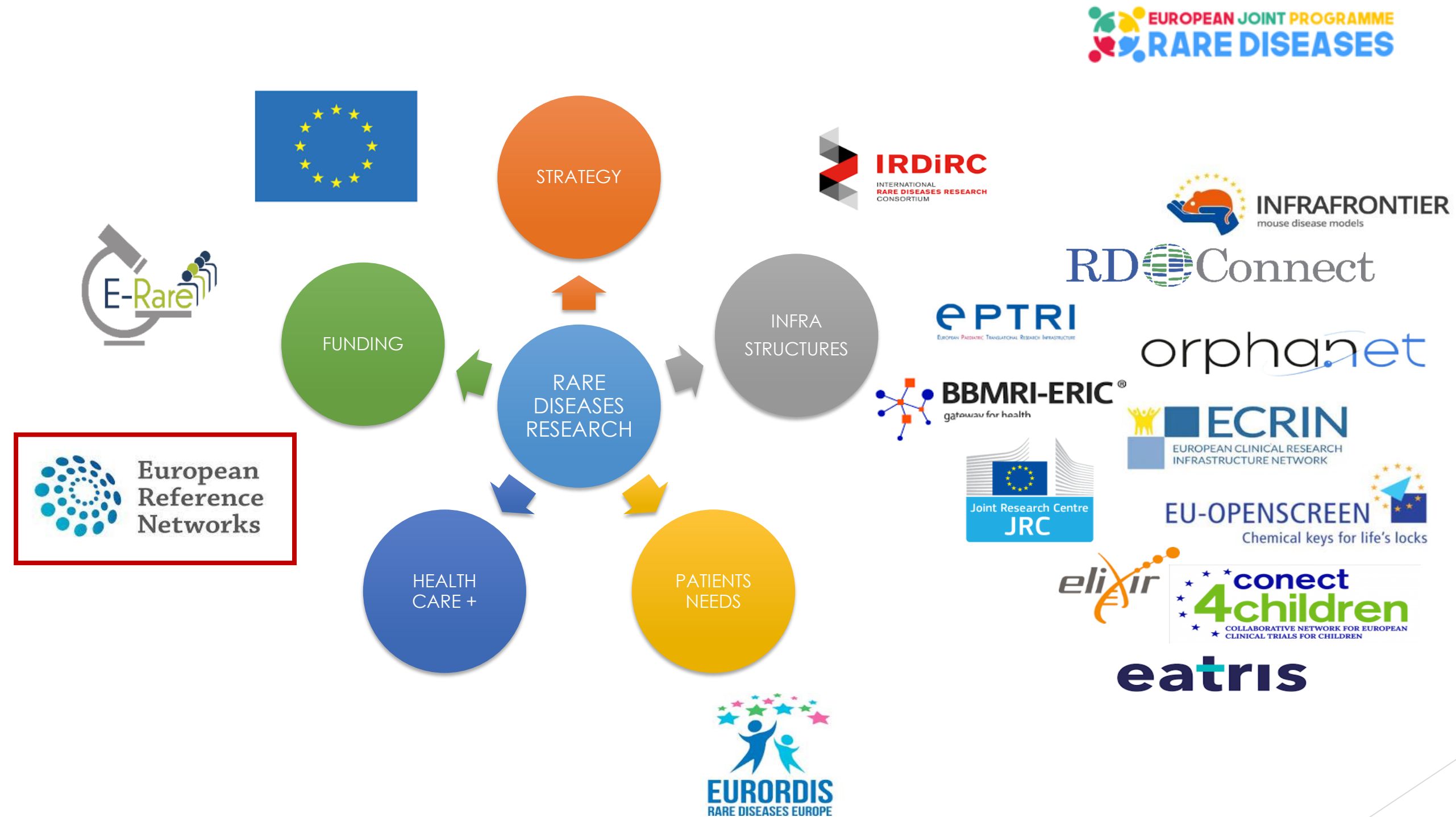
INSERM, US14 – Orphanet



Funded by
the European Union

ERICA has received funding from the European Union's Horizon 2020
research and innovation programme under grant agreement No 964908

Rare Diseases Landscape in Europe

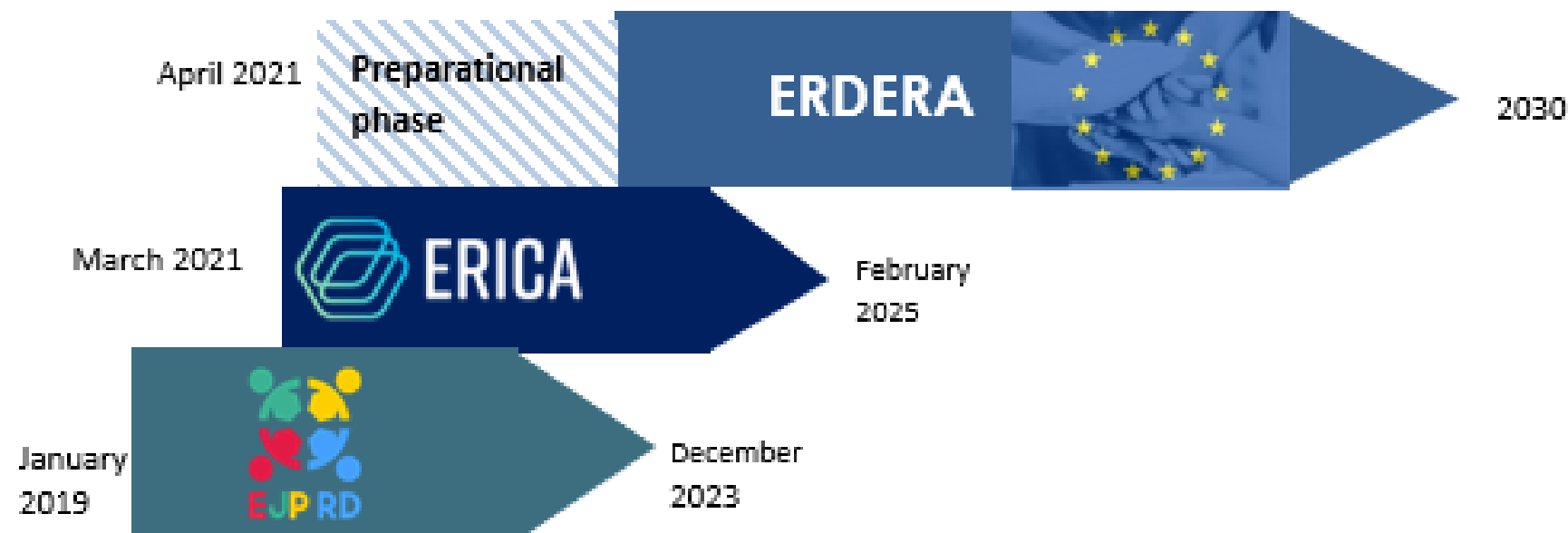


Courtesy of Dr. Daria Julkowska

Objectives



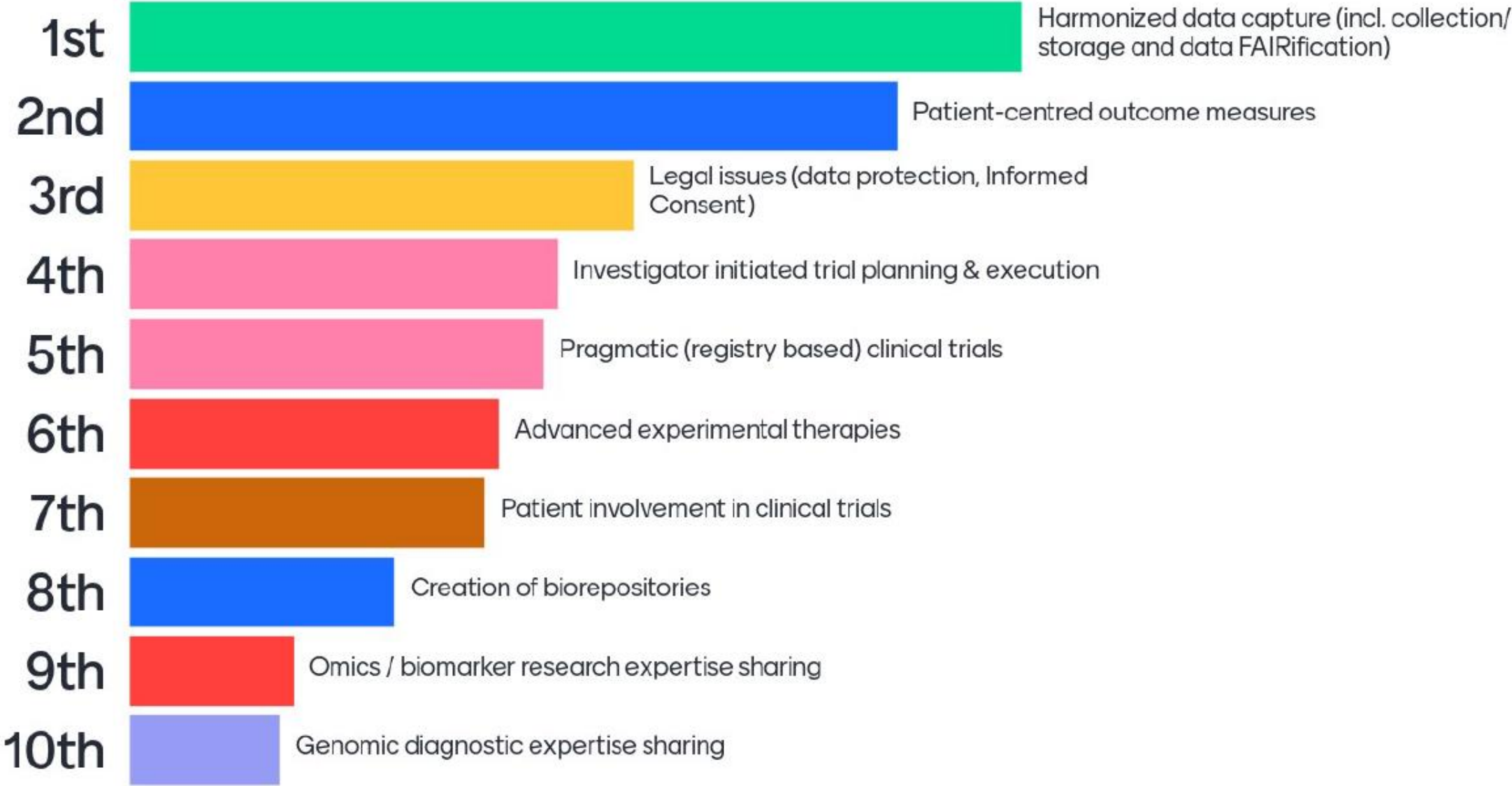
- The aim of ERICA, in which all 24 ERN's take part, is to strengthening ERN's research and innovation capacity of, through:
 - **facilitating inter-ERN collaboration**
 - **increase the visibility and impact of ERNs**
(outreach and collaboration with multiple stakeholders)
 - To align the strategy of ERICA and to **integrate the results into the new The European Rare Diseases Research Alliance (ERDERA)** to further stimulate the international collaboration and interoperability in RD Research and Clinical Network.



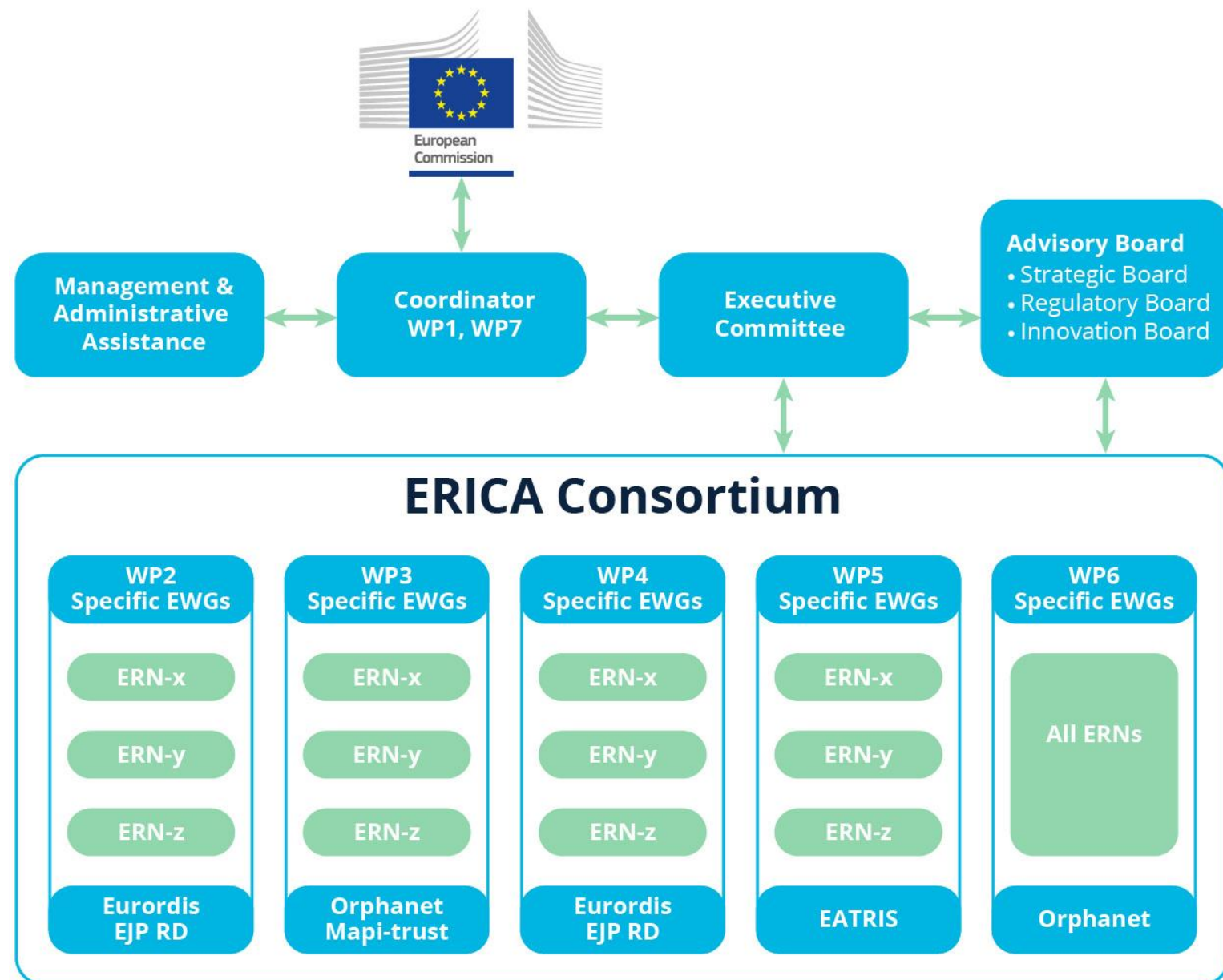
Goals of ERICA

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

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



Governance structure and structural operational framework



Alberto Pereira	WP1, WP6, WP 7 leader
Franz Schaefer	WP2 leader
Eduardo Lopez Granados	WP2 leader
Mar Mañú Pereira	WP3 leader
Ana Rath	WP3 leader
Ralf-Dieter Hilgers	WP4 leader
Luca Sangiorgi	WP4 leader
Ruth Ladenstein	WP5 leader
Anton Ussi	WP5 leader
Maurizio Scarpa	WP6 leader



Alberto Pereira



Franz Schäfer



Eduardo Lopez Granados



Mar Mañú Pereira



Ana Rath



Ralf-Dieter Hilgers



Luca Sangiorgi



Ruth Ladenstein



Anton Ussi

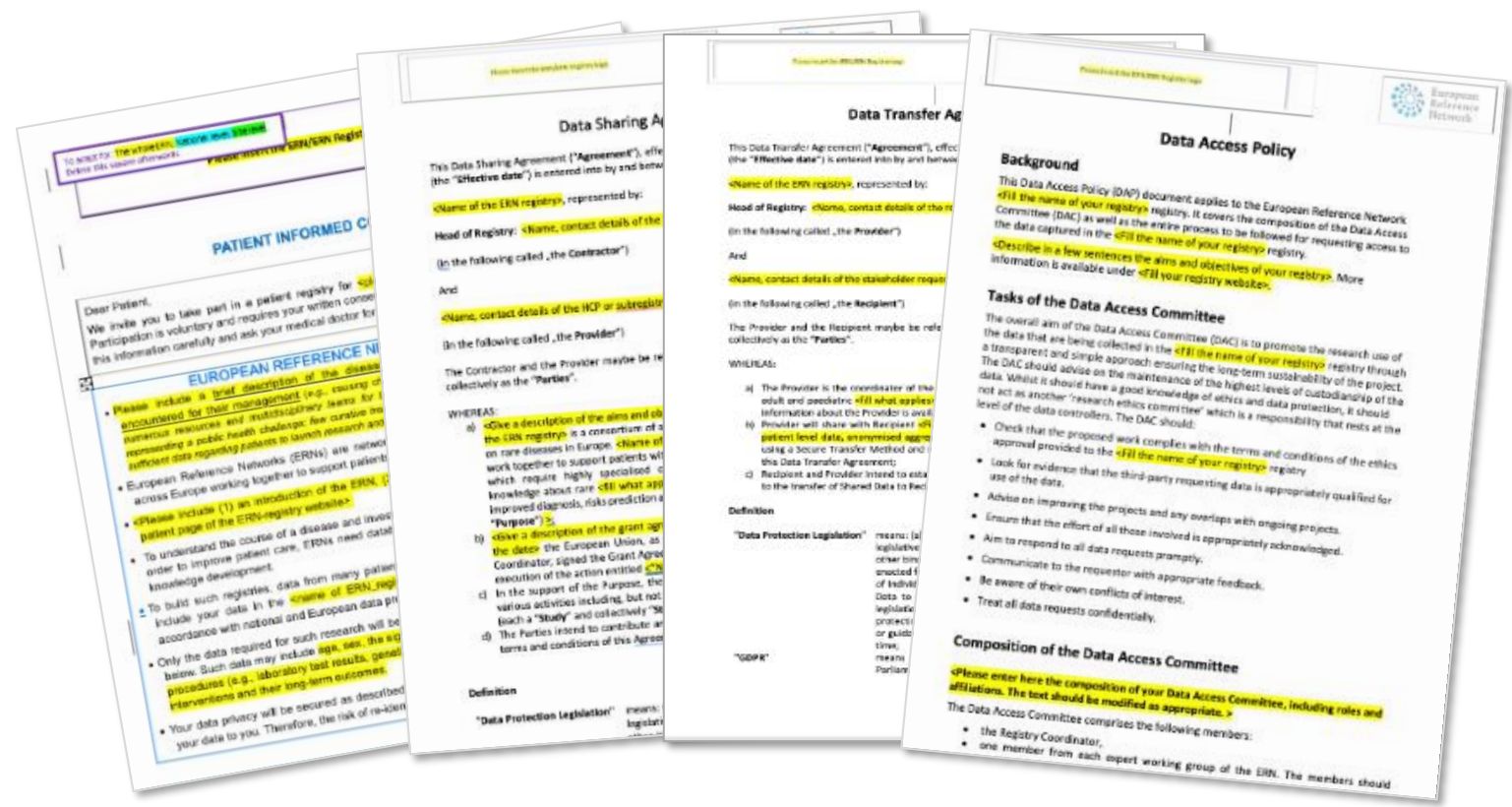


Maurizio Scarpa

WP2 **Data Collection, Integration and Sharing**

- WG Data collection
- WG Legal aspects
- WG Interoperability
- WG Secondary data use

WP2
Data Collection,
Integration and
Sharing



Common consent form based on ontologies

Program title
Horizon 2020

Start date of project
01 March 2021

Project number
964908

Duration
48 months

Project full title
the European Rare disease Coordination and Support Action

Project acronym
ERICA

Project coordinator
Prof. A.M. Pereira, Leiden University Medical Center

Deliverable N°: D2.3
Title: Recommendations on registry data collection

WP N° and Title: WP2 – Data Collection, Integration and Sharing

Lead beneficiary: UK-HD

Type: Report

Dissemination level: Confidential, only for members of the consortium

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PROMs Repository

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](#)

PCOM/PROM Name	Type	PROQOLID™	Age	Domains	Disease (OrphaName)	OrphaCode	Group of Diseases	ERNs	PROQOLID™ Link
Adult Sickle Cell Quality of Life Measurement Information System® (ASCQ-Me®)	PRO	✓ Full	Adult	- Emotional impact (20 items) - Social functioning (17 items) - Pain (13 items) - Stiffness (15 items) - Sleep functioning (12 items)	Sickle cell anemia	ORPHA:232	Rare anemia	ERN EuroBloodNet	Link
EORTC QLQ - Chronic Myeloid Leukaemia (EORTC QLQ-CML24)	PRO	✓ Full	Adult	- Symptom Burden - Impact on Daily Life - Impact on Worry/Mood - Body Image Problems - Satisfaction with Care and Satisfaction with Social Life	Non-Hodgkin lymphoma	ORPHA:547	Tumor of hematopoietic and lymphoid tissues	ERN EuroBloodNet ERN PaedCan ERN EURACAN	Link
EORTC QLQ - Non Hodgkin Lymphoma Low Grade Module (EORTC- QLQ-NHL-LG20)	PRO	✓ Full	Adult	- Symptom burden due to disease and/or treatment (4 items) - Physical condition/Fatigue (4 items) - Emotional impacts (4 items) - Worries/Fears health and functioning (8 items)	Non-Hodgkin lymphoma	ORPHA:547	Tumor of hematopoietic and lymphoid tissues	ERN EuroBloodNet ERN PaedCan ERN EURACAN	Link

Free Member Access for ERN members

Requests via info@erica-rd.eu



Search databases: COA, disease, drug, author, etc.

[My Searches](#) [Advanced search](#)



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- [COLLABORATIONS](#)
- [CATALOG](#)
- [SUBMIT A REQUEST](#)

Home > Instruments > DEBQ-C - Dutch Eating Behavior...

Dutch Eating Behavior Questionnaire - Child version (DEBQ-C)

van Strien T; Oosterveld P

- > Basic description
- > Contact and conditions of use
- > Languages
- > Descriptive information
- > Content validity documentation
- > Measurement properties
- > References and websites

Last update: February 2023

BASIC DESCRIPTION

- Published in 2008

Authors
van Strien T; Oosterveld P

Copyright
Instrument copyrighted by Hogrefe Publishing

Objective
To assess restrained, emotional, and external eating in 7- to 12-year-old children

Population of development

- Disease(s):** [+](#)
 - Feeding and Eating Disorders
 - Obesity

Type of Clinical Outcome Assessment (COA)

PRO

Original language(s)

- Dutch for the Netherlands

Translations
5 translation(s)

Bibliographic reference(s) of the original questionnaire
van Strien T, Oosterveld P. The children's DEBQ for assessment of restrained, emotional, and external eating in 7- to 12-year-old children. Int J Eat Disord. 2008 Jan;41(1):72-81 ([PubMed abstract](#))

WP4
Clinical Trial Support



European Rare Disease Research Coordination and Support Action

Educational Webinar 3 **WP5**

Bridging the gap between promising preclinical data and a successful Clinical Trial




Gilles Vassal
Institut Gustave Roussy, ACCELERATE



Joanne Lee
Newcastle University


Thursday, October 19, 2023 16:00 -17:00 CET



European Rare Disease Research Coordination and Support Action

Join our next webinar **WP4**

REMEDIA4ALL / drug repurposing and clinical trial readiness



Anton Ussi
EATRIS

October 2nd, 2023 13:00-14:00 CET



European Rare Disease Research Coordination and Support Action

Join our next webinar **WP4**

Introduction on Patient-Reported Outcomes and considerations before including them in a clinical trial.

Study Case from the ERN-EuroBloodNet



Céline Desvignes-Gleizes
Mapi Research Trust



Andreas Glenthej
Copenhagen University Hospital



Dora Peereboom
Patient Advocate

May 10th, 2023 12:30-13:30 CET



European Rare Disease Research Coordination and Support Action

Join our next webinar **WP4**

Framework for Patient Engagement in Clinical Trials



Virginie Hivert
EURORDIS - Rare Diseases Europe



Maria Cavaller Bellaubi
EURORDIS - Rare Diseases Europe

Wednesday, May 24, 2023 15:00 -16:00 CET

<https://erica-rd.eu/events/webinars/>



The graphic is a dark blue rectangular panel with white and light blue text. At the top left is the ERICA logo and text. At the top right is the full name of the action. Below this, the title 'Educational Webinar 2' is shown in light blue, followed by a green 'WP5' badge. The main title 'Practical advice on how to use the Catalogue of Services and the IMT' is in white. Three speaker portraits are shown in a row, each with their name and affiliation below. At the bottom, a calendar icon indicates the date 'October 24th, 2022' and an alarm clock icon indicates the time '13.00-14.00h CET'.

ERICA European Rare Disease Research Coordination and Support Action

Educational Webinar 2 WP5

Practical advice on how to use the Catalogue of Services and the IMT

Anton Ussi EATRIS

Agustin Arasanz Duque VHIR/EATRIS

Rosan Vegter EATRIS

October 24th, 2022 13.00-14.00h CET

<https://erica-rd.eu/event/wp5educational-webinar-2-practical-guide-on-how-to-use-the-catalogue-of-services-and-the-imt/>

Welcome at the ERICA website

The aim of the **European Rare Disease Research Coordination and Support Action** consortium (ERICA), in which all **24 European Reference Networks (ERNs)** take part, is to build on the strength of the individual ERNs and create a platform that integrates all ERNs research and innovation capacity.

Through knowledge sharing, engagement with stakeholders in the rare disease domain and assembly of transdisciplinary research groups working across the global health spectrum, ERICA strives to reach the following goals:

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

ERICA will strengthen research and innovation capacity by integration of ERN research activities, outreach to European research infrastructures to synergistically increase impact, and innovation. This will result in efficient access and safe therapies for the benefit of patients suffering from rare diseases and complex conditions.




Joint HMA/EMA Multi-stakeholder workshop on Patient Registries 12&13 February 2024

The Joint Heads of Medicines Agencies (HMA)/European Medicines Agency (EMA) Multistakeholder workshop on Patient Registries will bring together representatives of registry holders, regulatory agencies, pharmaceutical companies, patients, healthcare professionals, academia, and health technology assessment bodies to address the following topics: Patient registries for rare diseases, data sharing, and patient involvement.



Presentations ERICA & EJP RD Joint Conference available!

The European Rare Disease Research Coordination and Support Action- ERICA and European Joint Programme of Rare Diseases (EJP RD) Joint Conference took place 21st November 2023 in Amsterdam University Medical Centre. Main topics: Examples of Synergies between both projects (ERICA & EJP RD) Collaboration of ERNs and non-ERNs researchers Preparing [...]

Other News

12th edition of the European Conference on Rare Diseases and Orphan Products (ECRD) The European Conference on Rare Diseases & Orphan Products (ECRD) is the event that allows the dialogue and collaboration of over [...]

[READ](#)

1st REMEDI4ALL International Drug Repurposing Conference 6-7 March, 2024 in Barcelona

Join the first international drug repurposing conference "Bridging Boundaries: Innovating, Connecting & Reshaping Drug Repurposing", #IDR24, co-organised by REMEDI4ALL, Beacon and [...]

[READ](#)

EJP RD Rare Disease Training: "Training on strategies to foster solutions of undiagnosed rare disease cases" REGISTRATION IS NOW OPEN here ONLINE REGISTRATION until 14 January 2024. Registration will remain open for the reserve list only until [...]

[READ](#)

ERN Clinical Trials Repository



orphanet

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](#)

Search Reset

Column Visibility ▼

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Showing 3 Clinical Trials (of 41 total)

Name of Study	ERNs	Type	Status	OrphaCode	Disease	Phase	Date	Link
A Phase II, Multicenter, Open-label, Non-comparative Study to Evaluate the Pharmacokinetics, Pharmacodynamics, and Tolerability of Osilodrostat in Children and Adolescent Patients With Cushing's Disease	Endo-ERN	Clinical trial	Ongoing	641613	- Endogenous Cushing syndrome	2	2021-04-28	Link
A Phase 3, Open-label, Randomized, Multicenter, 12 Months, Efficacy and Safety Study of Weekly MOD-4023 Compared to Daily Genotropin - Therapy in Pre-pubertal Children With Growth Hormone Deficiency	Endo-ERN	Clinical trial	Ongoing	631 467	- Non-acquired isolated growth hormone deficiency - Non-acquired combined pituitary hormone deficiency	3	2017-10-31	Link
REAL4: A Trial Comparing the Effect and Safety of Once Weekly Dosing of Somapacitan With Daily Norditropin® in Children With Growth Hormone Deficiency	Endo-ERN	Clinical trial	Ongoing	631	- Non-acquired isolated growth hormone deficiency	3	2019-05-20	Link

<https://erica-rd.eu/work-packages/integration-outreach-dissemination/clinical-trials/>

Linked to 



REAL4: A Trial Comparing the Effect and Safety of Once Weekly Dosing of Somapacitan With Daily Norditropin® in Children With Growth Hormone Deficiency


Type of network : **Multinational clinical trials**

Geographic coverage : **Global**

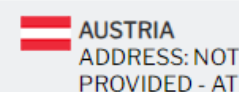
Funding body(ies) :-

Sponsor : **NOVO NORDISK A/S**

[Website](#) 

Study involving ERN members in at least two Member States 

Participants

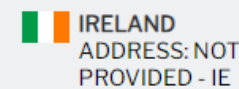


AUSTRIA
ADDRESS: NOT PROVIDED - AT

[REAL4: A Trial Comparing the Effect and Safety of Once Weekly Dosing of Somapacitan With Daily Norditropin® in Children With Growth Hormone Deficiency \(Phase 3\) - AT](#)

Institution: Information not provided - AT

[More information](#)



IRELAND
ADDRESS: NOT PROVIDED - IE

[REAL4: A Trial Comparing the Effect and Safety of Once Weekly Dosing of Somapacitan With Daily Norditropin® in Children With Growth Hormone Deficiency \(Phase 3\) - IE](#)

Institution: Information not provided - IE

[More information](#)



SPAIN
ADDRESS: NOT PROVIDED - ES

[REAL4: A Trial Comparing the Effect and Safety of Once Weekly Dosing of Somapacitan With Daily Norditropin® in Children With Growth Hormone Deficiency - ES](#)

Institution: Information not provided - ES

[More information](#)

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

- The ERNs harbour great research and innovation capacity, and ERICA has shown to be instrumental to map and identify this
 - The most eminent research topics for ERN have been identified and guide ERICA WP activities. Specific deliverables include, amongst others, the ERICA Research Wall, the ERICA Clinical trials Repository, and the ERICA PROMS Repository
 - Fostering of collaborative research projects enables to take advantage of:
 - the power of the ERNs’ large patient cohorts
 - the unique research expertise gathered in the EJP RD, IRDiRC, EMA, C4C, SolveRD, etc.
- New emerging intra- and inter-ERN rare disease competitive networks need further guidance and support
- The development of ERDERA, and the Clinical Research Network in particular, will enable to further integrate all required expertise to perform diagnostic- and outcome research, and develop innovative therapies, with clinical trials that use the required methodology for (ultra) rare diseases



ERICA

European Rare Disease Research
Coordination and Support Action

Thank you!

On behalf of the ERICA consortium

